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National Heart Transplantation Study

Preface

Detailed Table of Contents

Executive Summary

Dr. Roger W. Evans, Project Director
Dr. Joel H. Broida, Project Officer

Battelle Human Affairs Research Centers
Seattle, Washington
May 1985

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FOR THE NATIONAL HEART TRANSPLANTATION STUDY

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ROGER W. EVANS, Ph.D.

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DEDICATION

TO THOSE WHO HAVE STRUGGLED TO MAKE TRANSPLANTATION POSSIBLE: PHYSICIANS, PATIENTS, AND THEIR FAMILIES

PREFACE

PREFACE

By any standard, the National Heart Transplantation Study was a massive undertaking; few studies have been as comprehensive. Despite this, there were relatively few problems associated with project coordination and administration. Everyone involved gave their utmost to assure the success of the study.

The Preface to a report such as this offers an opportunity to reflect on the accomplishments of a unique group of researchers. The National Heart Transplantation Study was truly collaborative in every respect. While it is characteristic for a Project Director or Principal Investigator to take the "lion's share" of the credit for the successful completion of a research project, this is frequently misleading. As I think back over the three years since we began the study, I would be remiss if I did not acknowledge the many contributions of my colleagues, for this report is a joint product of many diverse minds. Perhaps I have served as the catalyst for our work or perhaps more appropriately the " social glue," but my colleagues and I have functioned as a team. The research scientists on the team have largely served as independent co-investigators with primary responsibility for their own areas of expertise--from conceptualization of the research through the preparation of the final report.

I pride my own autonomy, as well as that of the researchers with whom I work. Thus, in an effort to acknowledge our unique contributions, the chapters of this report have been organized according to subject area. These subject areas are largely consistent with the

seven components of the study--need estimates, donor organ availability, patient survival, patient quality of life, cost, and legal and ethical issues. The title page of each chapter often contains the name of one author. The name or names of the persons included on the title page are those persons who had primary responsibility in the writing of the chapter. This often, although not always, reflects major responsibility for the overall research reported in the chapter.

Unfortunately, this identification of authors tends to de-emphasize the very important role played by research assistants, research associates, project consultants, programmers, secretaries, and others who are integral to the research enterprise. They too are members of the team and their contributions are central to the successful completion of the research. Therefore, the first page of each volume of this report contains a complete list of all persons who have served on the research team for the National Heart Transplantation Study, regardless of their status on the team. Chapter 1 of the report identifies the role played by each individual.

The National Heart Transplantation Study has not been an easy study to conduct. On more than one occasion it has taxed us to the limit. We have been consumed by the task before us and frequently found the workload overwhelming. Perhaps the most significant problem was the collection of vast amounts of primary data, a task that would have been impossible to accomplish without the cooperation of the participating heart transplant programs, organ procurement coordinators, government officials, and various subcontractors. It is

simply impossible to mention everyone who has assisted us, but many names immediately come to mind--Mr. Carl Josephson, Dr. Joel Broida, Dr. Bryan Luce, Mr. Arne Anderson, Dr. Peter Frommer, Mr. Spencer Schron, Dr. Bernard Gersh, Dr. Myron Genel, Mr. Jerold Mande, Dr. Christopher R. Blagg, Dr. William Baumgartner, Dr. Bruce Reitz, Mr. Gene Pierce, Dr. Thomas L. Marchioro, Mr. Michael Shapiro, Dr. John J. Crowley, and, of course, the patients and families that chose to participate in the study.

Perhaps the greatest debt we owe is to the transplant teams that permitted us to look into the necessarily curious world of heart transplantation. At the University of Arizona we are especially grateful for the assistance we received from Dr. Jack Copeland, Ms. Jan Copeland, and Ms. Mary Jean McAleer. At the Columbia Presbyterian Medical Center (New York) our thanks go to Dr. Keith Reemtsma, Dr. Eric Rose, Ms. Joann Lamb, Ms. Laura Seche, and Ms. Fiorella Vataj. At the Medical College of Virginia our gratitude is extended to Dr. Michael Hess, Dr. Andrea Hastillo, and Ms. Margy Goldman. At the University of Minnesota we were particularly grateful for the assistance of Dr. R. Morton Bolman, III, Dr. Marilyn Strand, and Ms. Barbara Elick. At the University of Pittsburgh our sincerest gratitude is extended to Dr. Henry T. Bahnson, Dr. Robert L. Hardesty, Dr. Bartley Griffith, Ms. Ann Lee, Mr. Henry Rakov, Ms. Toni Talarico, Mr. Richard O'Brien, and Ms. Marianne Haramic. Finally, at the Stanford University Medical Center we are most appreciative of the assistance of the entire cardiac transplant team, but particularly, Dr. Stuart W. Jamieson, Dr. Philip Oyer, Ms. Patricia Gamberg, Ms.

Lois Takaoka, Ms. Polly Worrell-Uphoff, Ms. Leslie Sullivan, Ms. Martha Bott, Ms. Nancy Braun, and Ms. Geri Derby.

In addition we are especially grateful for the assistance we received from members of the Technical Review Committee--Dr. Marie Blackman, Mr. David McKusick, Dr. Thomas C. Schelling, Dr. Laurence Tancredi, Dr. Robert Wallace, and Dr. Leroy Walters.

I have undoubtedly left out the names of many others who have assisted us one way or another over the past three years. I apologize to those whom I have omitted.

As noted above, the report has been organized according to subject areas. It is also true that we have attempted, with limited success, to make each chapter of the report self-contained. In other words, we have written most of the chapters so that they stand alone from the complete report. As a result, some chapters appear redundant, in part, with other chapters. Our intent was to make it easy for people to gain access to the major findings in a particular subject area without necessarily reading the complete report. It was easier to accomplish this for some chapters than for others. To the extent that we were successful, we have added to the length of the report.

The report has been organized into five volumes. Volume 1 contains several chapters that provide a historical perspective and a description of the study methodology. Volume 2 contains those chapters addressing the need for heart transplantation and the supply of donor organs. Volume 3 is a rather complicated volume containing chapters on patient survival and quality of life, as well as a discussion of the cost of heart transplantation. Volume 4 is

concerned with the legal and ethical issues surrounding heart transplantation. Finally, Volume 5 contains chapters addressing a variety of miscellaneous issues as well as a bibliography on heart transplantation.

Finally, I would like to acknowledge that the role of Project Director is very demanding, often in ways that most would not recognize. At times these demands have been scientific, and at others they have been political. It has not always been easy to reconcile these demands as they have often been conflicting. It is in this regard that it has never been my intent to take a definite stand on any issue or to side with any particular interest group. In short, to my utmost ability, I have tried to remain objective. In doing so, however, I have come to know my weaknesses as a scientist. Unfortunately they are many. But the thanks goes to my colleagues, for they clearly provided the strength I needed as first a scientist and second a project director.

Roger W. Evans, Ph.D.
Project Director,
National Heart Transplantation Study
September 12, 1984
Mercer Island, Washington

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EXECUTIVE SUMMARY

A. INTRODUCTION

In 1980, the Health Care Financing Administration (HCFA) was posed with a major dilemma (Evans, 1983a). It had to decide whether or not heart transplantation should be a benefit paid for under the Medicare program. As administrator of the Medicare program, HCFA is routinely asked to make such judgments concerning the payment of health care technologies. These technologies, including surgical procedures (e.g., organ transplants), drugs (e.g., cyclosporine), and medical devices (e.g., insulin pumps), may be emerging (e.g., artificial heart), new (e.g., liver transplants), or existing (e.g., percutaneous transluminal coronary angioplasty). Payment decisions are commonly referred to as coverage determinations or coverage decisions. Within the Medicare program, coverage refers to benefits available to eligible beneficiaries. These decisions are more critical than they may first appear since coverage of a technology can markedly affect both its use and diffusion. For example, coverage of heart transplantation may well lead to an increase in the number of procedures performed as well as the number of hospitals that perform them. In short, existing transplant programs may increase their volume, while new programs will find the economic incentives offered by coverage to be attractive.

Once HCFA makes a favorable coverage decision, it also makes a concomitant decision commonly referred to as a reimbursement decision. This decision, unlike a coverage decision, is limited only to the amount and methods of payment for covered services.

Various governmental agencies are routinely called upon by HCFA to provide assistance in making coverage determinations. These include, but are not limited to, the National Institutes of Health and the Office of Health Technology Assessment within the National Center for Health Services Research. While these agencies provide assistance, they do not retain responsibility for the final coverage determination. HCFA simply uses the input of these agencies to make its final decision. Occasionally it is recognized that the scope of technology assessments required by HCFA to make coverage determinations are too broad to be addressed intramurally. As a result, it is sometimes necessary to use extramural research funds to conduct the required research through the grant or contract mechanism. Such has been the case with the National Heart Transplantation Study.

It is noteworthy that coverage decisions have been largely based on an assessment of the health effects of the particular technology that is in question. In other words, the research is often too narrowly focused. Also, many assessments have not been rigorous and the Office of Technology Assessment (1984) estimates that only 10 to 20 percent of the technologies used in medical practice have been shown to be efficacious by controlled clinical trials. In addition, and perhaps most unfortunately, the evaluation of nonmedical effects, such as economic and social effects, of specific technologies are usually not part of an assessment for coverage purposes. This has not been the case with the National Heart Transplantation Study, however.

Finally, it is notable that HCFA has never intended to interfere directly with the practice of medicine and the delivery of medical care. To minimize the possibility of this, HCFAs approach to the coverage determination process has been largely decentralized. As noted by the Office of Technology Assessment (1984), some Medicare coverage determinations are made at the national level in HCFAs central office, but most decisions are made by Medicare contractors who perform the Medicare program's claims processing and payment functions under HCFAs policy and operational guidance. Medicare contractors, often referred to as intermediaries and carriers, are either Blue Cross and Blue Shield plans or commercial insurers. On the U.S. mainland there are approximately 90 intermediaries administering Part A (institutional services) of the Medicare program, and 60 carriers that administer Part B of the program (physician's services). HCFAs ten regional offices assist contractors and transmit information between HCFAs central office and Medicare contractors.

Medicare coverage policy has not yet reached a steady state because it is developed and implemented in a decentralized manner. As noted by the Office of Technology Assessment (1984), national policy has developed largely as a result of questions from individual contractors about whether they should pay for specific technologies. Table ES-1 shows the sources of coverage questions submitted to HCFAs central office over a five year period. As shown here, the majority of these questions are relayed from HCFAs regional offices to the national office. HCFA periodically informs contractors about the coverage status of some specific technologies through transmittal

Table ES-1
Sources of Coverage Questions Submitted to
HCFA's Central Office

	PERCENT OF INQUIRIES BY SOURCE PER YEAR				
SOURCE	1978	1979	1980	1981	1982
HCFA regional office	61.3	32.1	59.4	34.6	24.1
Manufacturers	0.0	0.0	3.1	25.0	10.3
Hospitals/clinics	3.2	3.6	0.0	1.9	3.4
Physicians	3.2	14.3	0.0	9.6	10.3
Medical schools	0.0	7.1	0.0	0.0	3.4
Legislatures	3.2	0.0	0.0	1.9	3.4
Professional societies	6.5	17.9	6.3	3.8	0.0
Blue Cross	0.0	3.6	6.3	3.8	3.4
Blue Shield	0.0	0.0	6.3	0.0	0.0
Blue Cross/Blue Shield	0.0	0.0	0.0	0.0	3.4
Private insurance	0.0	0.0	3.1	3.8	0.0
Health Care Financing Administration	0.0	17.9	3.1	3.8	24.1
Public Health Service	0.0	0.0	6.3	3.8	6.9
Attorneys	0.0	0.0	3.7	0.0	3.4
Information not available	22.6	3.6	3.1	7.7	6.9
TOTAL	100.0	100.0	100.0	98.2	99.6

SOURCE: Office of Technology Assessment, 1984:79.

letters and a Medicare manual. However, as noted by the Office of Technology Assessment (1984), HCFAs coverage instructions have no standing in law or regulation, so the contractor's compliance is essentially voluntary.

It is readily apparent that there is considerable variation among Medicare contractors in terms of their coverage policies. These are largely attributable to the fact that HCFA coverage determinations have traditionally been based on what is considered "reasonable and necessary" for the care of the patient. Title XVIII of the Social Security Act specifies that this is to be the basis for coverage determinations. Section 1862 of the Act, among other things, prohibits payment by Medicare for any items or services which are "...not reasonable and necessary for the diagnosis or treatment of illness or injury or to improve the function of a malformed body member..."

In rendering coverage determinations, HCFA has instructed its contractors that they should observe a specific set of guidelines. These guidelines require that a technology must be:

- generally accepted as safe and effective
- not experimental
- medically necessary, and
- provided according to accepted standards of medical practice in an appropriate setting.

In general, coverage determinations are made at the local rather than the national level. According to the Office of Technology Assessment (1984), less than one percent of the 250 million claims

processed in fiscal year 1983 were sent to HCFAs central office for coverage decisions. Most questions are not of national interest. Additionally, contractors are concerned with processing claims as quickly as possible and view the coverage determination process as an unduly delay.

The Office of Technology Assessment (1984) has recently completed an extensive survey which shows that: "...when considering coverage questions, most medical consultants to contractors appear to rely on similar sources of information, including HCFA regional offices, colleagues in insurance companies or Blue Cross/Blue Shield plans, and state or national medical specialty societies." The results of the Office of Technology Assessment (1984) survey are shown in Table ES-2.

B. THE NATIONAL HEART TRANSPLANTATION STUDY: A SOURCE OF INFORMATION FOR COVERAGE POLICY

As stated by the Health Care Financing Administration, the purpose of the National Heart Transplantation Study was to "...examine, in general, all aspects of heart transplants, including the scientific, social, economic, and ethical issues, and, in particular, the impact of a possible Medicare decision to pay for heart transplants on the Medicare program, Medicare beneficiaries, and providers of health care." That heart transplantation became the focus of such an intense investigation is not surprising. In 1979, Department of Health and Human Services (DHHS) officials discovered that Blue Cross of Northern California, the regional Medicare intermediary, had paid for 23 transplants and 21 posttransplant cases at Stanford University Medical Center since 1973. Under the assumption that the procedure was

Table ES-2
Sources of Information Used By Medical Consultants
in Making Coverage Decisions

SOURCE	PERCENT OF CONSULTANTS USING SOURCE	
	YES	NO
Colleagues in another insurance company or Blue Cross/Blue Shield Association plan	78.6	21.4
HCFA regional office	87.5	12.5
University medical center	44.6	55.4
PSRO (PRO)	26.9	73.1
National insurance association	37.5	62.5
State or national medical or specialty association	75.0	25.0
Drug or device manufacturer	53.6	49.6
Other	51.0	49.0

SOURCE: Office of Technology Assessment, 1984:78.

experimental rather than therapeutic (Knox, 1980), DHHS attempted to cease payments for heart transplants but was forced to confront the issue. In doing so, the Department ultimately succumbed to political pressures and decided to pay for heart transplants at Stanford on a tentative or temporary basis, indicating that it would arrive at a final decision in early 1980.

On January 21, 1980, the National Institutes of Health recommended that Medicare pay for heart transplants performed at Stanford and other U.S. centers that "...met comparable standards of expertise, resources, and commitment, and for patients who fit the Stanford selection criteria, or 'acceptable equivalents'." The Secretary of the Department of Health and Human Services took issue with this recommendation because she felt many questions remained unanswered. In particular, she expressed concern about the patient selection process.

During this same period a controversy arose concerning the payment for a heart transplant performed at the University of Arizona in 1979. Blue Cross of Arizona, the Medicare intermediary locally, denied the University of Arizona's claim for the transplant because the operation was considered "experimental or investigational." In March, 1980 an administrative law judge ruled that Medicare must pay for the heart transplant.

As a result of the fear that additional claims for heart transplants would be forthcoming, Medicare stated that it would not pay for heart transplants, but that an extensive study would be undertaken on the subject. In doing so, the Secretary announced on

June 12, 1980 that DHHS would require new technologies to pass muster on the basis of their "social consequences" before "financing their wide distribution." In considering heart transplantation, the Secretary declared that it held a more or less symbolic place in the ranking of therapies with regard to aggressiveness and that it should serve as "the prototype" of technology assessment. As summarized by one observer (Knox, 1980), the proposed study of heart transplantation represented "...the first time in the 15-year history of Medicare the government has attempted to define what is meant by the statutory requirement that the program pay only for 'reasonable and necessary' medical care--including such controversy-fraught issues as 'necessary for whom' and 'reasonable under what circumstances'?"

On September 30, 1981, the Battelle Human Affairs Research Centers (Seattle, Washington), a division of the Battelle Memorial Institute (Columbus, Ohio), was awarded the contract to conduct the National Heart Transplantation Study by the Health Care Financing Administration. Dr. Roger W. Evans, the Project Director, was to be assisted by several staff members in conducting the study. The study was originally scheduled to be completed on February 28, 1983.

Twelve medical centers applied to participate in the study, in essence serving as demonstration sites. These institutions were to be reimbursed for a total of 15 heart transplants. Of the institutions that applied, six were chosen by HCFA to participate in the study. These institutions are as follows:

- University of Arizona Health Sciences Center
- Columbia Presbyterian Medical Center

- Medical College of Virginia
- University of Minnesota
- University of Pittsburgh
- Stanford University Medical Center

Each of these institutions was required to meet a very specific set of criteria and comply with the study protocol. Each institution was required to furnish information about heart transplants performed during a period starting no later than January 1, 1975 and continuing through the period of the study. This included data on each institution's facility and personnel resources, heart donor program, patient selection criteria, transplant and patient care protocols, patient follow-up care, patient survival, cost of establishing and maintaining a heart transplant program, patient charge information, and other similar and related information. Ultimately data were compiled on 441 heart transplant cases since 1968. (Of these patients, 152 living patients provided in-depth data on their experience.)

The total cost of the study (excluding the heart transplants) has been $1.6 million. The total period of performance has been 35 months. The study as completed is more comprehensive than originally envisioned by HCFA. It is noteworthy, however, that the final report for the National Heart Transplantation Study is completely devoid of recommendations. The Health Care Financing Administration retains sole responsibility for policy formulation.

C. THE HISTORY AND CURRENT STATUS OF HEART TRANSPLANTATION

While developments in heart transplantation cannot be viewed apart from developments in organ transplantation more generally, it is impossible to review all the landmark events in either experimental or clinical heart transplantation. The first experimental cardiac transplantation was reported in 1905 by Carrel and Guthrie of the Hull Physiological Laboratory at the University of Chicago. In their early work, Carrel and Guthrie removed hearts from puppies and sutured them into the necks of adult mongrel dogs.

The first clinical or human heart transplantation was performed on December 3, 1967 by Christiaan Barnard in Capetown, South Africa. Barnard's first patient lived 18 days before succumbing to a combination of infection and rejection. Barnard performed his second heart transplant on January 2, 1968. This patient lived nearly 18 months before dying of coronary artery insufficiency due to chronic rejection.

In the U.S., the first heart transplant was performed on December 6, 1967 by Adrian Kantrowitz at Maimonides Hospital in Brooklyn, New York. The patient was 17 days old and died after 6.5 hours. The second heart transplant was performed by Norman Shumway at the Stanford University Medical Center on January 6, 1968. This patient died after 15 days. By December, 1970, fifty-four teams accounted for 73 of the 167 transplants recorded by the National Heart Institute. Two teams, those of Shumway and Denton Cooley (Texas Heart Institute) had done more than 25 percent of these procedures. After the initial enthusiasm for heart transplantation had subsided, only two teams

continued to be active--that of Shumway at Stanford and Richard Lower at the Medical College of Virginia. Stanford continues to be the most active heart transplant program in the world (see Table ES-3).

Table ES-4 shows the number of heart transplants performed each year in the U.S. The marked increase in 1980 roughly coincides with the introduction and use of cyclosporine (a new immunosuppressive drug) on heart transplant recipients. The Stanford group initiated their cyclosporine trial in December, 1980. The University of Pittsburgh began its trial in March, 1981. By March, 1983 nearly all active heart transplant programs in the U.S. were using cyclosporine.

There are currently about 20 heart transplant programs in the U.S. In 1983, 13 of these programs, including the McGuire VA Hospital in Richmond, Virginia, performed 172 heart transplants and 13 heart-lung transplants (see Table ES-5). As described below, it is likely that over the next five years there will be a substantial increase in the number of institutions performing heart transplants. Nonetheless, there are several constraints on the overall level of heart transplant activity including: (1) cost and reimbursement, (2) the number of centers performing them, and (3) the acute shortage of donor organs.

D. RESULTS

The National Heart Transplantation Study was intended to address numerous complex and controversial issues. In recognition of this, the study was organized into several major analytic components. These included: (1) an epidemiological or need analysis, (2) a survival

Table ES-3
Number of Heart Transplants At Stanford By Year

YEAR	NUMBER OF PROCEDURES*
1968	10
1969	9
1970	8
1971	12
1972	14
1973	15
1974	15
1975	19
1976	21
1977	20
1978	31
1979	25
1980	22
1981	25
1982	28
1983	39

*Excludes heart-lung transplants.

Table ES-4
Number of Heart Transplants Performed By Year, U.S.

YEAR	NUMBER OF PROCEDURES PERFORMED
1967	1
1968	54
1969	34
1970	16
1971	13
1972	14
1973	19
1974	21
1975	23
1976	24
1977	23
1978	34
1979	32
1980	36
1981	62
1982	103
1983	172
TOTAL	681

Table ES-5
Number of Heart Transplant Procedures By Institution, 1983

INSTITUTION	NUMBER OF HEART TRANSPLANTS	NUMBER OF HEART-LUNG TRANSPLANTS
Stanford University Medical Center*	39	7
University of Pittsburgh*	29	5
Medical College of Virginia (and VA)*	20	
Columbia-Presbyterian Hospital*	26	
Texas Heart Institute	16	
University of Minnesota*	14	
University of Alabama	11	
University of Arizona*	10	
Methodist Hospital (Indianapolis)	7	
St. Louis University Hospital	6	
Johns Hopkins	4	1
Mayo Clinic	0	—
TOTALS	172	13

*Participant in the National Heart Transplantation Study.

analysis, (3) an organ procurement analysis, (4) a cost analysis, (5) a sociomedical (quality of life analysis), (6) a legal analysis, and (7) an ethical analysis. However, the analyses contained in the final report are not limited to just these topics. For example, an attempt has been made to examine the potential diffusion of heart transplantation among hospitals with open-heart surgery facilities. The issue of payment for heart transplant procedures is discussed at great length. In addition, numerous implications of heart transplantation are considered. For example, the approach followed in conducting this study of heart transplantation is described as prototypical to health care technology assessment more generally. Also, within the report, an approach to resolving the experiment-therapy dilemma is presented and the implications of the artificial heart are considered. Finally, two more difficult issues are addressed, namely, lessons learned from the treatment of end-stage renal disease, and the problems associated with health care technology and resource constraints.

As in all studies, it is important to gain some perspective on the persons being studied. This presentation, therefore, begins with a brief description of heart transplant recipients based on patient interviews and the abstraction of medical and financial records. This discussion is then followed by a topic-by-topic summary of the study results.

1. PATIENT DESCRIPTION

The average age of living heart transplant recipients in this study was 42.4 years (S.D. = 9.99). The youngest patient was 19 years of age and the oldest was 60. The majority (87.5 percent) of living

heart transplant recipients are male. Patients are predominantly white (87.5 percent) and married (73.0 percent). Over 52 percent of the patients have attended college or have college degrees.

With regard to medical considerations, the mean length of time since diagnosis of heart disease for living recipients is 5.4 years (S.D. = 7.53). The primary disease diagnosis is idiopathic cardiomyopathy (50.7 percent) followed by coronary artery disease (37.3 percent). Living heart transplant recipients exhibited relatively few contraindications prior to heart transplantation, whereas deceased patients were much more likely to have had contra-indications. Not surprisingly, the majority (81.3 percent) of patients had New York Heart Association Class IV function at the time of evaluation for a heart transplant. At the present time, 88.0 percent of the living patients have Class I function. Of the living patients, 50.7 percent are currently on cyclosporine as their primary immunosuppressive agent, 99.3 percent are receiving prednisone, and 43.9 percent, azathioprine. In contrast, 14.6 percent of the deceased patients were on cyclosporine at the time of their death, 85.4 percent on prednisone, and 63.0 percent on azathioprine. Nearly 50.0 percent of the deceased patients died prior to discharge from the hospital.

Both infection and rejection are important considerations. The primary agents of infection have been herpes simplex and cytomegalovirus (CMV). Overall, living heart transplant recipients experienced an average of 2.03 infectious episodes, deceased patients experienced 3.81 episodes, and all patients, combined, experienced 2.50 episodes. Of the living heart transplant recipients, 17 (11.2

percent) had experienced no rejection episodes. The majority (63.8 percent) have experienced between 1 and 3 rejection episodes. Living heart transplant recipients experienced an average of 2.46 (S.D. = 1.94) rejection episodes, while deceased patients experienced an average of 2.26 (S.D. = 2.03) episodes.

Given time constraints, tissue matching has not been a primary consideration in heart transplantation. In all cases, ABO blood group compatibility and a negative crossmatch between recipient serum and donor lymphocytes is sought prior to transplantation. Among living heart transplant recipients, in 64.6 percent of the cases, the results of the lymphocyte crossmatch were unknown. Only three patients were known to have a positive crossmatch. The most HLA antigens any patient matched with their donor on is 6. The majority of patients matched on 0 or 1 antigen.

Comorbidity among organ transplant recipients is an important consideration both before and following transplant surgery. The five most prevalent complications among heart transplant recipients posttransplant, excluding patients who had the conditions both pre- and posttransplant, are (1) osteoporosis (18.2 percent), (2) diabetes (14.9 percent), (3) vision disorders (14.3 percent), (4) back or spine disorders (8.8 percent), and (5) cancer (6.1 percent). All these complications are known to be associated with immunosuppressive therapy.

There are many social and family considerations relevant to the heart transplant experience. In general, heart transplant recipients had close family and social relationships. This is not

surprising since social support is of primary importance as a patient selection criterion. Most patients appear to have adjusted quite well to their transplant, although many indicated that the transplant has been a source of concern and problems. However, half the living recipients noted that they were "very happy" these days. Most patients appeared to be realistic in their assessment of their health status and their level of physical functioning is consistent with their health status evaluations. Most patients showed marked improvement in their functional ability when comparisons are made before and after their heart transplant (see Table ES-6). About 30 percent of the patients indicated that they enjoyed nonstrenuous activities during the course of a week. Forty percent of the patients characterized themselves as "not very active, sitting and walking mostly." Half of the patients said they are now doing most of the things they were doing before their illness, but even so, 50 percent of the patients said they were unable to work because of their health. Approximately 30 percent of the recipients of heart transplants are currently working.

Many patients have concerns related to finances, although the household income of the majority of patients is $15,000 or more. Nearly two-thirds of the patients currently receive Social Security retirement or disability benefits and another 17 percent receive union or employer disability benefits (see Table ES-7). While private insurers have helped transplant recipients meet their medical expenses, nearly 70 percent of all patients indicated that they still have

Table ES-6
Limitations Heart Transplant Patients Experienced One-Year Before
Their Heart Transplant And Those Experienced Currently
(Expressed As A Percent)

BECAUSE OF HEALTH DID/DOES PATIENT	TIME PERIOD	
	BEFORE	NOW
Need assistance traveling around community	32.9	8.6
Stay indoors most or all of the day because of health	41.4	6.6
Stay in bed most or all of the day because of health	41.4	6.6
Limited in vigorous activities	75.7	70.4
Trouble walking several blocks or climbing stairs because of health	73.0	34.2
Trouble bending, stooping, or lifting because of health	63.8	43.4
Trouble walking one block or climbing one flight of stairs because of health	62.5	16.4
Help in walking such as another person, cane, crutches, etc.	17.8	7.2
Unable to do certain amounts of work, housework, or schoolwork	71.1	52.6
Unable to work at job or do work around house	63.8	47.4
Need help eating, dressing, bathing, or using toilet	13.8	1.3
Limited in any way from doing anything he wants to	73.0	66.4

Table ES-7
Sources of Income Support For Living Heart Transplant Recipients

SOURCE OF INCOME SUPPORT	NUMBER AND PERCENT RECEIVING ASSISTANCE	
	NUMBER	PERCENT*
Social Security Retirement or Disability Benefits	95	62.5
Supplemental Security Income	5	3.3
Railroad Retirement Benefits	0	0.0
Veterans Administration Benefits	13	8.6
Unemployment Compensation	0	0.0
Workmen's Compensation	1	0.7
Aid to Families With Dependent Children	3	2.0
Civil Service Benefits	1	0.7
Union or Employer Disability Benefits	27	17.8
Union or Employer Retirement or Pension Benefits	7	4.6
Other Benefits	14	9.2

*Does not total 100 percent because a person could receive benefits from more than one source.

out-of-pocket expenses (see Table ES-8). About 60 percent of the patients indicated that they were extremely or moderately concerned about finances.

2. THE NEED FOR HEART TRANSPLANTATION

For more than 40 years, diseases of the heart and blood vessels have been the major cause of death in the United States. It is estimated that 42,750,000 Americans have one or more forms of heart or blood vessel disease. In 1981, 984,610 people died with cardiovascular disease. Over one-fifth of all persons killed by cardiovascular disease are under age 65. It is often assumed, incorrectly, that many of these people would benefit from heart transplantation. One of the primary objectives of the National Heart Transplantation Study was to estimate how many people in the U.S. each year might benefit from heart transplantation. To accomplish this, separate morbidity and mortality analyses were undertaken.

Over the past decade, the criteria for selecting heart transplant recipients have remained very restrictive. In general, patients must be under age 55 and be free of comorbid conditions (e.g., insulin-dependent diabetes mellitus) that are likely to diminish the chances of a successful transplant. Current patient selection criteria are provided in Table ES-9. Undoubtedly, if current patient selection criteria are relaxed, the number of potential recipients would increase substantially, and could include people who are less sick than those currently selected to undergo cardiac transplantation.

Table ES-8
Sources of Payment For Medical Expenses Incurred By Living Heart Transplant Recipients According to Transplant Period (Expressed As A Percent)

	TRANSPLANT PERIOD*		
SOURCE OF PAYMENT	PRETRANSPLANT	TRANSPLANT	POSTTRANSPLANT
Out-of-Pocket	67.8	40.8	68.4
Private Insurance	71.7	66.4	73.7
Medicare	15.1	14.5	32.3
Medicaid	5.9	5.3	9.2
Transplant Center	19.7	53.3	40.8
Direct Donations	27.0	16.4	12.5
CHAMPUS Program	.7	.7	.7
Veteran's Administration	7.2	3.9	5.9
Other	7.9	3.3	4.6

*Totals exceed 100.0 percent because a person could have multiple sources of payment.

Table ES-9
Contraindications To Heart Transplantation As Specified By the National Heart, Lung, and Blood Institute's Special Advisory Group

(1) Advancing age--e.g., beyond the age (normally about 50) at which the individual begins to have diminished capacity to withstand postoperative complications;

(2) Severe pulmonary hypertension as reflected, for example, by a pulmonary artery systolic pressure over 65-70mm Hg and exceeding pulmonary artery wedge pressure by about 40 or more mm Hg, or a calculated pulmonary vascular resistance above approximately 6 Wood units (applicable to orthotopic cardiac transplantation because of the limited work capacity of a normal donor right ventricle);

(3) Irreversible and severe hepatic or renal dysfunction (because of the likelihood of exacerbation early postoperatively and because of interference with immunosuppressive regimens);

(4) Active systemic infection (because of the likelihood of exacerbation with initiation of immunosuppression);

(5) Any other systemic disease considered likely to limit or preclude survival and rehabilitation after transplantation;

(6) A history of behavior pattern or psychiatric illness likely to interfere significantly with compliance with a disciplined medical regimen (because a lifelong medical regimen is necessary, requiring multiple drugs several times a day with serious consequences in the event of their interruption or excessive consumption);

(7) Recent and unresolved pulmonary infarction or pulmonary roentgenographic evidence of abnormalities of unclear etiology (because of the likelihood of pulmonary infection or its exacerbation with initiation of immunosuppression under such circumstances);

(8) Insulin-requiring diabetes mellitus (because of exacerbation by chronic corticosteroid therapy);

(9) Symptomatic or documented severe asymptomatic peripheral or cerebrovascular disease (because of observed accelerated progression in some patients after cardiac transplantation and on chronic corticosteroid treatment);

Table ES-9 (Continued)
Contraindications To Heart Transplantation As Specified By the National Heart, Lung, and Blood Institute's Special Advisory Group

(10) Acute peptic ulcer disease (because of the likelihood of early postoperative exacerbation);

(11) The absence of adequate external psychosocial supports for either short or long-term bases (because such support is generally necessary during the inevitable waxing and waning of the clinical status of the patient and for adherence to the lifelong medical regimen).

SOURCE: Federal Register Vol. 46, No. 14, Pgs. 7072-7074, Thursday, January 22, 1981.

Several previous attempts have been made to measure the need for cardiac replacement. In some instances these estimates have been for heart transplantation, in others for the totally implantable artificial heart, and in still others, for left ventricular assist devices, whether these be for short- or long-term use. As shown in Table ES-10, these estimates, based on varying assumptions, have differed greatly from report to report.

In the National Heart Transplantation Study, several sources of data have been used to derive another set of need estimates. These data include: (1) the National Hospital Discharge Survey data (to estimate prevalence), (2) national mortality data (to estimate deaths), and (3) data from the Medicare Statistical System (to estimate need among current Medicare beneficiaries). Each of these data sources has various limitations that make them less than ideal for estimating need. Nonetheless, they remain the primary data that are available for this purpose.

Since most data sets available for producing need estimates are based on primary disease diagnosis, the first step in the analysis was to identify those conditions that might render a person eligible for a heart transplant. This task was accomplished in consultation with a group of cardiologists and cardiac transplant surgeons very familiar with the indications for heart transplantation.

As shown in Table ES-11, for only three years--1977, 1978, 1979--are estimates available from each data source for both the morbidity and mortality need indicators. This is because data for the most recent five years were obtained for the analyses. No attempt was

Table ES-10
Previous Estimates of the Need For Cardiac Replacement

PANEL OR GROUP	DATE OF REPORT	ESTIMATED NUMBER
Ad Hoc Task Force to NIH	1969	11,736 - 32,168
Artificial Heart Assessment Panel NHI	1973	16,750 - 50,300
Stanford (John Schroeder)	1979	75,000
Russell and Cosimi	1979	10,000 - 50,000
NHLBI Advisory Council	1980	8,000 - 15,000
Lubeck and Bunker	1982	16,000 - 66,000
Stanford (Pennock et al.)	1982	1,000 - 5,000

Table ES-11
Summary of Need Estimates According to Need Indicator
For Persons 10-54 Years of Age

NEED INDICATOR	YEAR 1975	1976	1977	1978	1979	1980	1981
	(NUMBER OF PERSONS)						
MORBIDITY--HOSPITAL DISCHARGE DATA							
U.S. POPULATION[a]	N/A	N/A	483,324	473,244	289,221	281,438	267,486
MEDICARE BENEFICIARIES ONLY[b]	22,915	26,315	28,425	28,585	20,240	N/A	N/A
MORTALITY--DEATHS							
U.S. POPULATION	N/A	N/A	24,179	23,941	13,980	14,085	N/A
MEDICARE BENEFICIARIES ONLY[b]	N/A	N/A	1,421	1,372	1,012	N/A	N/A

[a]All estimates based on age and diagnosis, excluding diabetics.

[b]Refers to current Medicare beneficiaries only. All estimates based on age and diagnosis, no other exclusions.

N/A = Not analyzed or not available.

made to assure complete overlap in years of data because of the desire to analyze the most recently available data. It is evident that there is considerable variation between estimates for 1977 and those for 1979. Rather than being indicative of a substantial decline in cardiovascular morbidity and mortality, this variation is largely attributable to the fact that the 1977 and 1978 data were coded according to the 8th Revision of the International Classification of Diseases (ICDA-8-REV), while the 1979, 1980, and 1981 data were coded according to the 9th Revision of the International Classification of Diseases (ICD-9-CM).

Using the most stringent indicator of the need for heart transplantation, that being mortality, Table ES-11 indicates that in 1980, approximately 14,100 people ages 10-54 years died from conditions for which heart transplantation may be indicated. Of these 14,100 people, the referral experience of the Stanford cardiac transplant program would suggest that, upon further evaluation, only 1,932 people or 13.7 percent of them would have been potentially accepted for a transplant. Of these 1,932 people, 1,177 or 60.9 percent would have been likely to receive a heart transplant. The remaining patients would have died awaiting a donor or would have continued to await the availability of one. If these same exclusion fractions are applied to the 1979 mortality data, it is expected that 1,167 patients would have been likely to receive a heart transplant.

Estimating the need for heart transplantation among current Medicare beneficiaries is very difficult--the data available to do so are quite sparse. Also, in order to qualify for Medicare benefits, a

person must be 65 years of age or older to have been declared disabled for a two-year period. Since being over the age of 55 is considered an absolute contraindication to heart transplantation, under existing entitlement rules, only disabled persons would be considered as potential heart transplant candidates. As shown in Table ES-12, the number of persons who are currently Medicare-eligible that are likely to receive a heart transplant is extremely small using the percentage exclusions derived from the application of strict patient selection criteria. Thus, while it is estimated that in 1979 only 1,012 Medicare beneficiaries between the ages of 10 and 54 years died of conditions for which heart transplantation may be indicated, it is expected that only 85 would have received a heart transplant. The remainder would have been excluded because of contraindications or would have died awaiting a donor heart.

Table ES-12 summarizes the foregoing estimates of the number of heart transplants that are likely to be performed in the U.S. according to two age groups--those 10-54 and those 10-49--for persons not eligible for Medicare benefits as well as those that are Medicare eligible. However, it must once again be emphasized that these estimates would change dramatically if the prevailing patient selection criteria are relaxed to accomodate sicker patients.

3. THE AVAILABILITY OF DONOR HEARTS

The number of available donor organs is a key factor in determining the number of heart transplants that are performed each year. Thus, providing a reasonable estimate of the potential availability of donor hearts was one of the major objectives of the

Table ES-12
Projected Number of Heart Transplants Among Non-Medicare and Currently Medicare-Eligible Persons, U.S.

POTENTIAL RECIPIENT CATEGORY	YEAR 1979	YEAR 1980
AGES 10-54		
NON-MEDICARE ELIGIBLES	1,167	1,177
MEDICARE ELIGIBLES	85	N/A
AGES 10-49		
NON-MEDICARE ELIGIBLES	636	650
MEDICARE ELIGIBLES	35	N/A

N/A = Not available.

National Heart Transplantation Study. Clearly, the number of suitable hearts available for transplantation is limited. Strict medical selection criteria, limitations in the long distance transportation of cadaveric hearts, lack of long-term methods of organ preservation, and the extensiveness of networks established for the procurement of donor organs are the major factors to be considered in estimating the potential supply of donor hearts. Another important consideration is the willingness of individuals to donate hearts and other organs for transplantation.

Estimates of the availability of donor hearts were made based on several data sources. These include:

- a telephone survey of a national probability sample of 2,056 respondents regarding their attitudes toward organ donation
- data on in-hospital deaths as reported by the National Hospital Discharge Survey for the years 1979-1981
- data on 1,955 organ donors obtained through a survey of 80 organ procurement programs across the U.S.

The approach which was used in estimating organ availability involved producing a range of estimates based upon these various data sources. The figures range from maximum estimates based on the total number of hearts available in the U.S. each year considering only the medical selection criteria imposed on heart donors, to much lower estimates based on the number that are likely to be available through existing organ procurement networks.

With respect to public attitudes toward organ donation, several interesting results were observed. While most people (93.7 percent) had heard about organ donation prior to the interview, less than 14 percent of all respondents indicated that they carried an organ donor card. Those respondents who said that they did not carry an organ donor card were asked if they would be prepared to sign and carry a donor card. Only 27.8 percent responded "Yes," while 53 percent said that they didn't know. This would suggest that many people are uncertain about organ donation. Thus, public education campaigns aimed at these individuals could perhaps increase the potential supply of donor organs.

Moreover, respondents were somewhat more likely to donate the organs of relatives than they were to donate their own organs. Almost 53 percent indicated that they would be willing to donate the organs of a relative who had just died, while approximately 50 percent said that they would be willing to donate their own kidneys and 46 percent said that they would be willing to donate their own heart. Since it is common practice to require family consent, even if a person has signed an organ donor card, the willingness of individuals to donate the organs of relatives is important in estimating organ availability.

Finally, the results of the survey seem to suggest that presumed consent, viewed by many as the best way of increasing organ availability, would not be very popular among the American public. An overwhelming 86.5 percent felt that doctors should not have the power to remove organs from people who had died and had not signed an organ donor card without consulting the next-of-kin. However, the majority

felt that if someone signs an organ donor card, the decision should not have to be approved by his or her next-of-kin.

The first and by far the most restrictive factor limiting the potential supply of donor hearts is the stringent set of medically related selection criteria imposed on heart donors. Since the donor heart must still be beating at the time of removal, all cardiac donors must meet an acceptable definition of brain death. Thus, only in-hospital brain dead patients are possible candidates for consideration as heart donors. Moreover, experience has shown that organs from male donors under the age of 35 and female donors under the age of 40 have the greatest likelihood of resulting in a successful transplant. In most cases death is the result of traumatic head injuries (i.e., blunt trauma, gunshot wound) or cerebrovascular accidents. An acceptable cardiac donor must have no history of cardiac disease or cardiac abnormalities and have no evidence of thoracic or cardiac trauma, including intracardiac drug administration during resuscitative procedures. A potential heart donor must have no history of transient, severe hypotension after injury. A donor must also be free of malignancy and infection at the time of death.

Hospital discharge survey data were used to estimate the maximum number of donor hearts that would be available in the U.S. each year considering only the medical selection criteria imposed on heart donors. These estimates were based on analysis of the National Hospital Discharge Survey data for the years 1979 through 1981--the most recent three years for which these data are available. The advantage of using the National Hospital Discharge Survey data, rather

than national mortality data, in estimating organ availability is that the National Hospital Discharge Survey data provide information on in-hospital deaths. The data file also contains information on patient characteristics which are necessary in determining whether or not a particular patient is a potential heart donor. More specifically, the data file contains both demographic (i.e., age, sex) and medical (i.e., primary and secondary diagnoses) variables which are useful in excluding patients as potential organ donors.

The procedures used in estimating the availability of donor hearts from the National Hospital Discharge Survey Data were relatively straightforward. First, all patients who were discharged from the hospital dead were selected for further analysis. Next, information regarding the age and sex of patients was used to reduce the number of potential heart donors. Only females 5 to 39 years of age and males 5 to 34 years of age were identified as potential donors. Estimates, however, were also made in which children 5 to 9 years of age were excluded from consideration as potential heart donors. Finally, specific illnesses and diseases which serve as contraindications to heart transplantation were identified and used to further reduce the number of potential heart donors.

Based on this analysis, it is clear that there is a maximum number of hearts that are available each year for transplantation. As can be seen from Table ES-13, considering current selection criteria, the maximum number of potential donors is approximately 16,000 (ranging from 13,896 in 1980 to 17,936 in 1981). This represents less than 2 percent of all in-hospital deaths. If children 5 to 9 years of

Table ES-13
The Estimated Availability of Donor Hearts Based on the Number of In-Hospital Deaths, and Modified by the Willingness of Individuals to Donate Organs

YEAR	ESTIMATE BASED ON IN-HOSPITAL DEATHS	ESTIMATE MODIFIED BY WILLINGNESS TO DONATE ORGANS*
1979	16,222	8,598
1980	13,896	7,365
1981	17,936	9,506

*Based on a Survey of Public Attitudes Toward Organ Donation conducted specifically for the National Heart Transplantation Study.

age are excluded as potential heart donors, the number is further reduced to approximately 15,000. Moreover, since only 53 percent of individuals indicate a willingness to donate the organs of a relative who had just died, perhaps at most only 7,000 to 9,000 hearts are available each year for transplantation.

In order to provide a more realistic estimate of the availability of cadaveric hearts, an estimate was made of the availability of hearts through the existing network of programs which were established for the procurement of kidneys and other organs. In order to make this estimate, it was necessary to obtain information on the characteristics of individuals who donate kidneys and other organs to determine the proportion that would have met minimal requirements for heart donation.

Data for this analysis were obtained from a survey of 80 organ procurement programs over an 8 month period of time. For each organ donor, programs were asked to provide information which included age, sex, primary cause of death, and heart function on arrival at hospital. Programs were also asked to provide information on infections, associated disease, and other contraindications to heart donation. Data on a total of 1,955 individuals who donated kidneys and other organs were collected and used to estimate the proportion of those organ donors that would have met the strict medical selection criteria imposed on heart donors. This information was then used to derive an estimate of the number of donor hearts that would be available based on the number of cadaveric kidney transplants that are performed in the U.S. each year.

The data indicate that based on the age criterion alone, approximately two-thirds (66 percent) of all organ donors are potential heart donors. However, of these donors, many exhibit one or more of the various contraindications to heart donation. Depending upon the specific contraindications used, only 15 to 40 percent of all kidney donors may be acceptable heart donors.

Table ES-14 presents estimates of the availability of donor hearts based on the number of cadaveric kidney transplants performed each year. The figures indicate that only a relatively small number of hearts are likely to be available through the existing network of organ procurement programs. In 1983, for example, of a total of 2,164 cadaveric donors, the number of available hearts could be as high as 866 or as low as 325.

The results of this analysis would suggest that organ procurement programs currently harvest a rather large proportion of the acceptable hearts from organ donors. Better communication among organ procurement programs regarding the need for extrarenal organs could perhaps result in a modest increase in the number of hearts harvested. Large increases in the supply of acceptable donor hearts, however, would require an increase in the total number of organ donors. Thus, programs aimed at increasing public awareness, as well as the awareness of the medical community, of the need for donor organs could potentially increase the willingness of individuals to donate organs for transplantation.

Another way to increase organ availability would be to change the donor selection criteria. Imposing less strict selection criteria

Table ES-14
Estimates of the Availability of Donor Hearts Based on the Number of Cadaveric Kidney Transplants Performed Each Year

YEAR	TOTAL NUMBER OF CADAVERIC KIDNEY DONORS*	LOW ESTIMATE OF AVAILABLE HEARTS**	HIGH ESTIMATE OF AVAILABLE HEARTS**
1980	1,711	257	684
1981	1,713	257	685
1982	1,846	277	738
1983	2,164	325	866

*Number derived by dividing total number of cadaveric kidney transplants performed by 2. It should be noted that these numbers assume that both kidneys were used. It also does not take into consideration a 20 percent wastage rate. In 1982, for example, it is estimated that there were approximately 2,200 donors.

**Low estimate based on 15 percent and the high estimate based on 40 percent of all kidney donors.

upon potential heart donors (i.e., increasing the allowable age or accepting donors with other medical contraindications) would increase organ availability. However, accepting less than ideal organs for transplantation could result in reduced transplant success and recipient survival rates.

It should, of course, be emphasized that a realistic estimate of donor availability is an extremely important component of a comprehensive study of heart transplantation. The number of heart transplants performed each year will most certainly influence heart transplant costs. Moreover, a large disparity between the supply of donor hearts and the need for heart transplants has definite legal and ethical implications. The importance of an accurate estimate of donor availability is underscored by the fact that, until a viable artificial heart is developed, the supply of acceptable donor organs is likely to be the major factor limiting the number of heart transplants that are performed each year.

4. SURVIVAL

Over the years there has been considerable debate concerning the survival of persons who receive heart transplants as well as those who do not. Many of the key issues involved are complex and remain unresolved. For example, in the statistical literature it has been argued that persons gain little net benefit from heart transplantation, once patient selection biases are taken into account. However, both the surgical and transplantation literature continue to refute this claim showing that transplant recipients survive in ever increasing numbers for extended periods of time.

Survival rates for one through five years have continued to improve, with the Stanford University Medical Center group now reporting one-year survival rates in excess of 80 percent and five-year survival rates in excess of 50 percent.

In attempting to resolve the major issues involved here, a multifocal approach to the analysis of survival data was followed in the National Heart Transplantation Study. As an initial step, it was important to determine if heart transplantation actually prolonged life, taking into consideration possible selection biases. A second step amounted to an examination of the relative risk of mortality within various subgroups of transplant recipients and for different transplant periods. Finally, survival curves were estimated for various subgroups of transplant recipients and across different transplant periods. The results of these analyses are highlighted below.

Given the complexity of the issues involved, the Cox proportional hazards model was used to analyze survival data for 414 heart transplant recipients in the study. The Cox model has two particularly attractive features. First, it is possible to control statistically for possible selection biases that may be introduced into the analyses, such as surgeons transplanting only healthier patients. Secondly, the Cox model is a multivariate method, unlike techniques such as that of Kaplan and Meier which is used to estimate a single survival curve for a homogeneous group of patients. Because of this, the Cox model makes it possible to control statistically for other confounding variables. These combined features render the Cox

model to be the most powerful statistical technique available for the analysis of survival data. In the absence of a controlled clinical trial, there is no other reasonable method to approach the issue of patient survival.

As noted above, an important step in the analyses was to ascertain whether transplantation actually prolonged life. In the literature, the argument is that only healthier patients receive heart transplants because sicker patients die before a donor heart becomes available. The suggestion is that a selection bias is introduced here that favors transplantation. As one author notes, this approach is problematic because it fails to "...make allowance for the fact that the treated patient must have at least survived from time of diagnosis to time of treatment while no similar requirement..." is made of the untreated controls. Despite this controversy, two major papers in the statistical literature have shown that, based on data from 1968 to 1974, there is some benefit associated with heart transplantation, especially for younger, healthier transplant recipients.

While there is clearly some potential for bias in the selection of patients for heart transplantation, the evidence presented in the surgical literature suggests that such bias may be random rather than systematic. If it is random, then the selection problem is less significant since random bias introduces fewer problems into the analysis of the data. In fact, it is likely that cardiac surgeons "stack the deck" against themselves since they allegedly choose the sickest rather than the healthiest patients to transplant when a donor heart becomes available.

The surgeons have shown rather persuasively that patients who are declared candidates but for whom a donor heart does not become available are unlikely to survive in excess of six months (see Figure ES-1). One report from the Stanford group indicates that 90 percent of these patients are dead within 3 months after selection. The average length of survival for these patients is said to be 45 days. In the National Heart Transplantation Study the average length of survival was 41 days for patients who failed to receive a transplant. Also, the post-transplant survival time of heart transplant recipients is depicted in Figure ES-2. Based on findings such as these, proponents of cardiac transplantation have argued that patients yield a net survival benefit.

A second step in the analysis of the survival data was concerned with the relative risk of mortality among various subgroups of transplant recipients and across various transplant periods. It was found that the relative risk of mortality for females was 1.3 times that of males, while whites had a relative risk that was 1.2 times that of nonwhites. These differences in relative risk, however, did not prove to be statistically significant. The relative risk of mortality associated with transplant period was found to decrease over time, with the least risk associated with the 1980-1983 period. These differences in relative risk were highly significant. The relative risk associated with both age at transplant and level of functional impairment were each found to be statistically significant. Younger patients were at lower risk of death as were patients who had less

Figure ES-1
Actuarial Survival Curves of Heart Transplant Recipients
And Those Candidates Who Were Selected For Heart
Transplantation But Who Died Before
A Donor Became Available

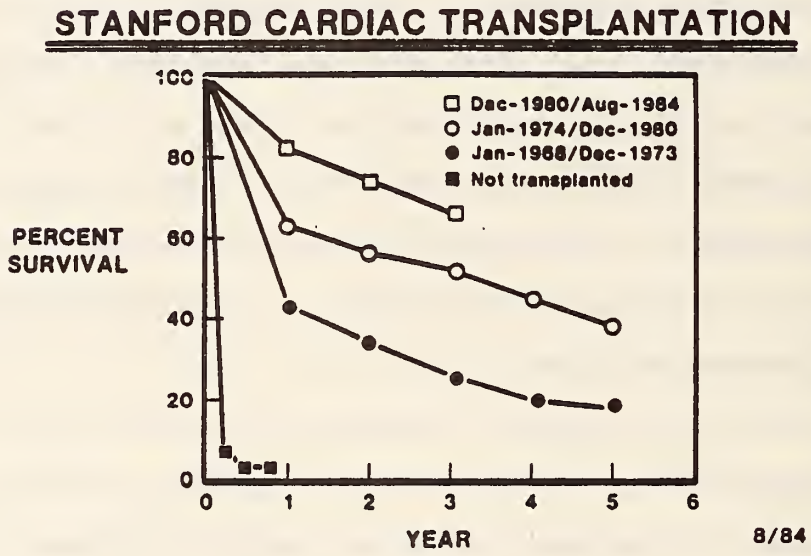
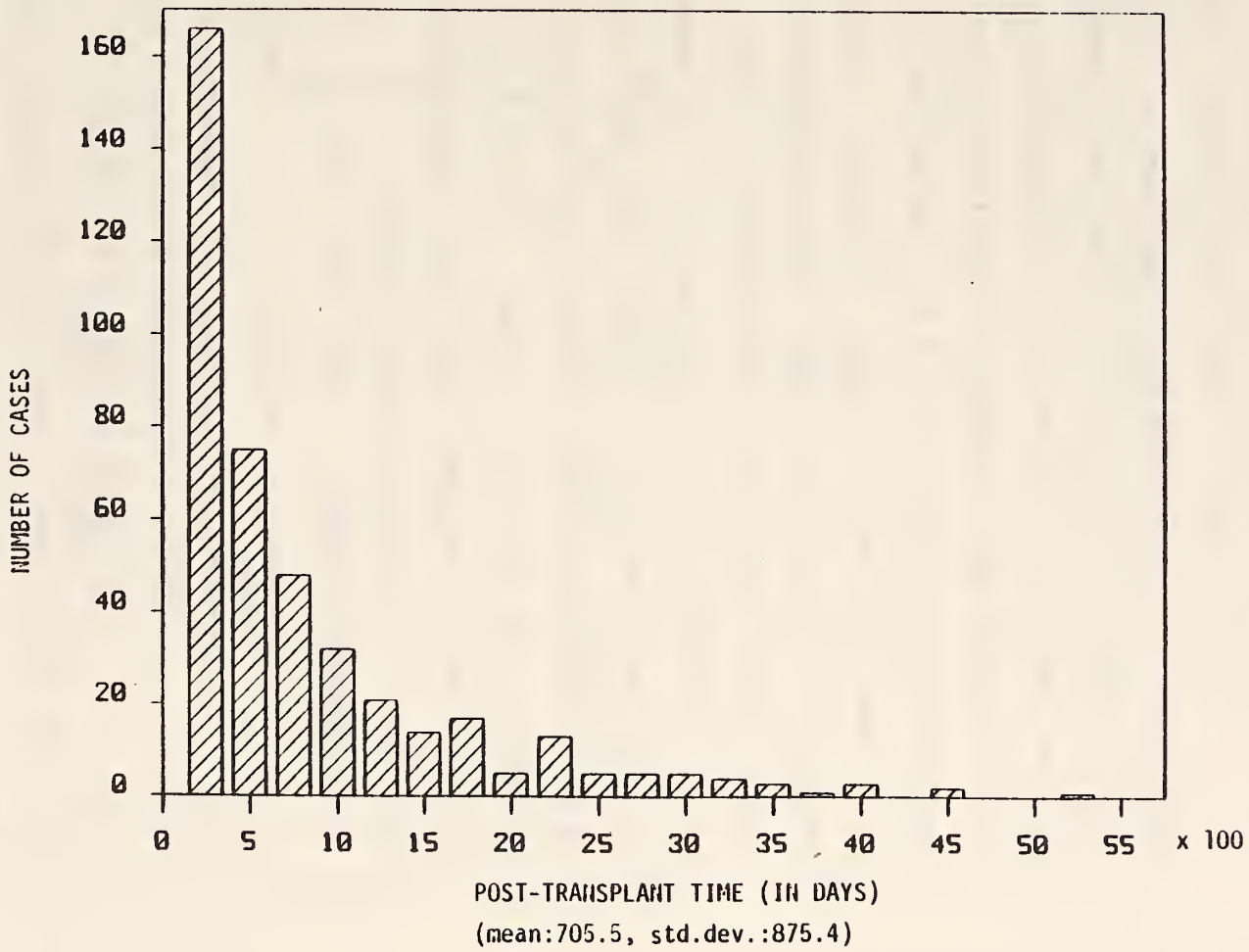


Figure ES-2
Post-Transplant Survival Time in Days For Heart
Transplant Recipients



functional impairment just prior to transplant. The results of the relative risk analyses are summarized in Table ES-15.

The final step in the survival analysis consisted of an examination of the survival curves generated by the Cox model for various subgroups of transplant recipients and at various transplant periods. The results revealed that the median length of survival for white recipients was 1000 days (2.7 years), while that for nonwhites was 1600 days (4.4 years). Males had a median length of survival of approximately 1000 days (2.7 years), and females, 600 days (1.6 years). The survival curves for white and nonwhite recipients indicated that the first 500 days after transplantation constitutes a high risk period, with mortality stabilizing thereafter. The projected survival for all heart transplant recipients shows that one-quarter will survive longer than 10 years. Finally, survival rates have improved considerably over the years as shown in Figure ES-3. In fact, the survival rate for the 1980-1983 period is approximately 25 percent better than for the baseline period, 1968-1973.

5. QUALITY OF LIFE

The assessment of quality of life is a complex matter. In fact, it is sometimes argued that it is impossible to meaningfully measure the quality of life of people in the general population, let alone that of people who are chronically or terminally ill. Nonetheless, the National Heart Transplantation Study provided a unique opportunity to make such an assessment.

Historically the success of medical interventions has been judged almost solely on the basis of survival. Today, the criteria

Table ES-15
Relative Risks of Mortality After Heart Transplantation in Relation to Various Potential Risk Factors: Results of Proportional Hazards Analyses Controlling For The Other Risk Factors

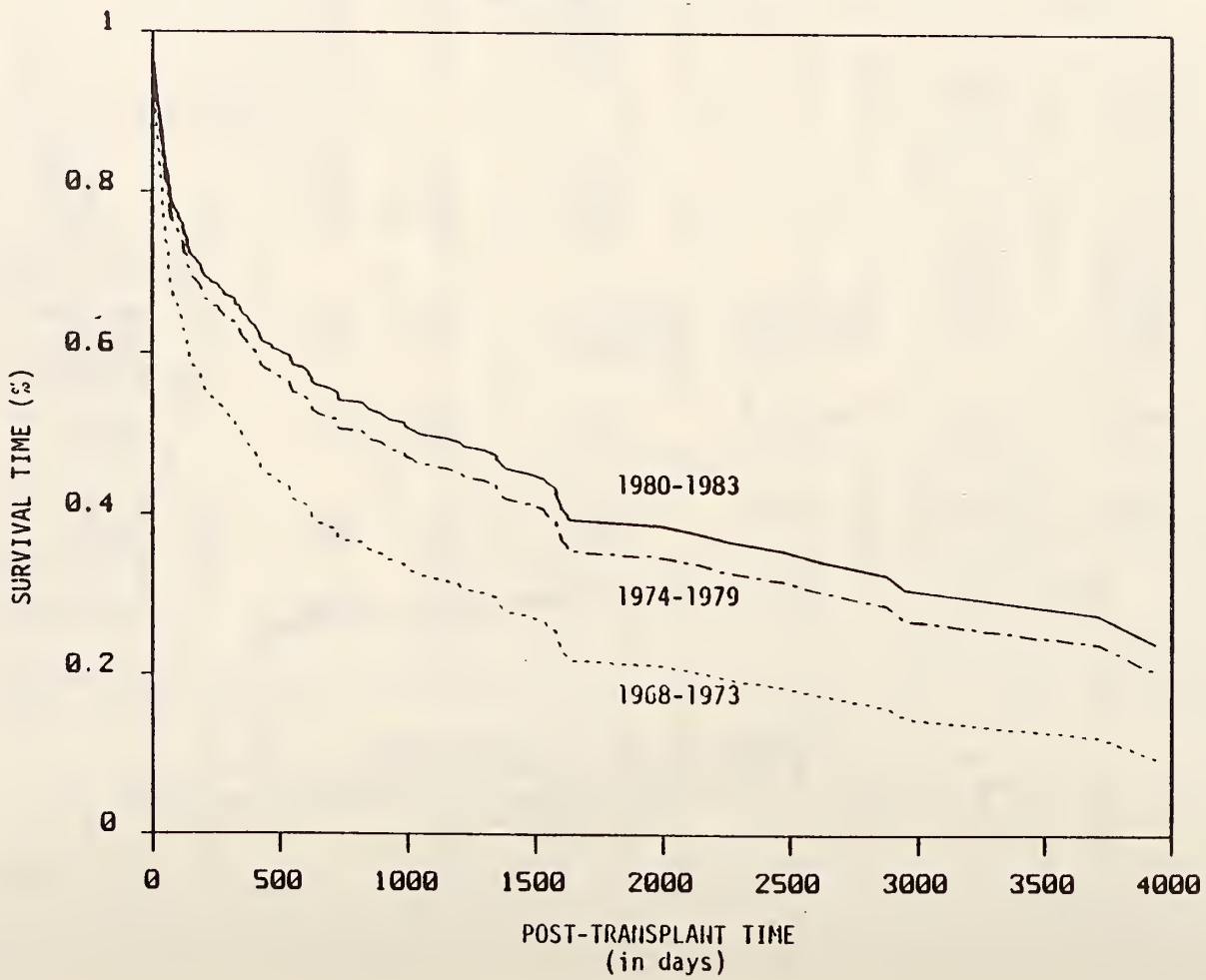
Risk Factors	Number of Recipients Dead	Number of Recipients Alive	Relative Risk	95%CI	$X^2_{(1)}$	p-value
Sex						
- Male	208	152	1.00			
- Female	34	26	1.30	0.896-1.904	1.90	0.25
Race						
- Nonwhite	15	20	1.00			
- White	224	158	1.21	0.727-2.016	0.54	0.25
Transplant Period						
1968-1973	58	5	1.00 (1.61)			
1974-1979	89	41	0.68 (1.47)	0.482-0.969	4.62	0.05
1980-1983	95	132	0.62 (1.00)	0.730-0.881	7.08	0.01
Age at Transplant[a]					10.50[c]	0.001
- 20	17	20	1.00			
20-20	23	33	1.25			
30-39	48	47	1.55			
40-49	103	65	1.93			
50+	51	13	2.41			
Functional Impairment[a, b]					10.69[c]	0.001
1	0	1	1.00			
4	5	8	1.57			
5	14	12	1.82			
6	29	26	2.13			
7	69	62	2.88			
8	38	15	3.35			
9	64	35	3.35			
10	22	17	3.89			

[a]Based on a restrictive proportional hazards model.

[b]Lower scores = fewer functional limitations (e.g., 1=normal; no complaints; no evidence of disease; 10=moribund, fatal processes progressing rapidly.

[c]Score test for linear trend.

Figure ES-3
Post-Transplant Survival Time In Days By
Three Transplant Periods For Heart
Transplant Recipients



for judging success often includes some attempt to characterize the degree to which patient outcomes other than survival, such as employment, functional ability, and life satisfaction, are either restored to pretreatment levels or at least are improved to a level that exceeds that at the time treatment was initiated. In short, people have come to expect more of medical treatment than mere survival.

Medical technological advances now sustain the lives of people that just a few years ago would have been impossible to sustain. Concomitantly, more frequently than ever before, clinicians and patients are forced to confront what has become known as the quality/quantity of life tradeoff. On the one hand, in rendering their judgments, clinicians can decline to offer patients available treatment if the patient is not expected to benefit. On the other hand, patients, upon being offered various therapies, may decline to accept them on the basis of the quality of life they may afford.

The same quality of life measures used in the National Heart Transplantation Study have been previously used in the National Kidney Dialysis and Kidney Transplantation Study (Evans et al., 1984). In the latter study it was determined that quality of life could be conceptualized as having two dimensions--one objective and one subjective. The objective quality of life indicators included: (1) functional impairment, (2) ability to work, and (3) health status. The subjective quality of life indicators included: (1) well-being, (2) psychological affect, and (3) life satisfaction. Standard measures were used to evaluate both dimensions of quality of life.

The quality of life of 152 living heart transplant recipients was studied intensively. All patients were asked to complete a questionnaire that included questions on all aspects of their life and, in particular, their transplant experience. In order to provide some basis for comparison, the responses of the heart transplant recipients have been compared with those of the 859 patients that participated in the National Kidney Dialysis and Kidney Transplantation Study.

Perhaps the most frequently analyzed patient outcome in studies of therapeutic intervention is work or employment status. Whether or not a previously working patient has been able to return to work is a primary consideration in the rehabilitation of the patient. In cardiovascular studies, return to work has been a particularly important goal and a frequent indicator of successful intervention.

Table ES-16 shows the employment status of the heart transplant recipients at four different time periods--historically, during the year prior to transplant, at the time of evaluation, and currently. As shown here, 79.5 percent of the patients have been employed either part-time or full-time, historically. During the year prior to transplant, this fell to 55.3 percent. At the time of evaluation only 8.1 percent of the patients were working. Finally, at the present time, 31.6 percent of the patients are working. Nonetheless, it appears that a larger percentage of patients feel they are able to work since 57.9 percent of the living patients said they could work either full-time or part-time.

For purposes of comparison, the results of the National Kidney Dialysis and Kidney Transplantation Study have been compared with

Table ES-16
Employment Status of Heart Transplant Recipients
At Different Time Periods
(Expressed as a Percent)

EMPLOYMENT STATUS	WORK STATUS HISTORICALLY[1]	DURING YEAR PRIOR TO TRANSPLANT[2]	AT TIME OF EVALUATION[1]	CURRENT WORK STATUS[2]
Employed Full-Time	78.3	46.1	6.7	25.0
Employed Part-Time	1.2	9.2	1.4	6.6
Employed But Temporarily Laid Off	0.0	0.0	0.5	0.0
Unemployed, Looking For Work	0.0	0.0	0.0	3.9
Unemployed, Not Looking For Work	0.2	0.0	0.7	0.0
Unable To Work Because of Health (Disabled)	3.8	33.6	80.7	50.7
Homemaker	4.7	3.3	3.0	3.3
Student (Full-Time)	11.6	5.3	3.5	2.0
Retired	0.0	2.6	2.8	7.9
Other	0.2	0.0	0.7	0.7

[1]All heart transplant recipients.

[2]Only living heart transplant recipients.

those of the National Heart Transplantation Study. These results for four time periods are provided in Table ES-17. As shown here, with the exception of baseline, kidney transplant recipients are more likely to be working at all time periods than any other patient group. They are generally followed by home hemodialysis patients, then heart transplant recipients, and then in-center hemodialysis patients.

As in all studies of the quality of life of patients undergoing medical interventions, differences in work status are undoubtedly due in part to factors other than treatment. For example, socio-demographic factors, lifestyle characteristics, and health status prior to treatment clearly can influence post-treatment outcomes. For example, heart transplant recipients, as a group, are older than kidney transplant recipients. Also, there is considerable evidence that prior to transplantation, heart transplant recipients are much sicker than either kidney transplant or kidney dialysis patients. Their life expectancy is usually only a few days. Thus, it is probably unreasonable to expect heart transplant recipients to return to work at the same rate as kidney transplant recipients. As shown in Table ES-17, heart transplant recipients compare more favorably with in-center hemodialysis patients. Of the dialysis patients, in-center dialysis patients tend to be sicker than other dialysis groups.

Clearly there are other aspects of quality of life that are as equally important as employment. Several of these aspects have been analyzed for heart transplant recipients as well as kidney transplant and kidney dialysis patients. The latter two patient groups provide a

Table ES-17
Work Status of Different Patient Groups At Different Time Periods

	PERCENT OF PATIENTS WORKING			
PATIENT GROUP	BASELINE[1]	1-YEAR	3-YEARS	5-YEARS
Heart Transplant Recipients	55.3	34.2	36.0	27.3
Kidney Transplant Recipients	64.6	49.1	51.8	49.2
Home Hemodialysis Patients	70.0	37.0	38.1	42.7
In-Center Hemodialysis Patients	67.1	19.5	18.9	14.9
Continuous Peritoneal Dialysis Patients	60.5	8.3	0.0	0.0
All Dialysis Patients	67.5	26.1	27.8	29.5

[1]Baseline equals during the year prior to transplantation.

basis for comparison. Without some standard for comparison, it is obviously difficult to interpret the quality of life indicators for heart transplant recipients. Also, to provide a better perspective on selected measures, the quality of life of heart transplant recipients is compared with that of the general population. The comparisons with the general population function as essentially the "norms" of comparison. This is not to say, however, that heart transplant, kidney transplant, and renal dialysis patients should be on equal footing with the general population with regard to quality of life.

Table ES-18 summarizes the results of the analysis of the three objective and three subjective quality of life indicators. A mean or average score and a standard deviation has been computed for each indicator. With the exception of the Karnofsky Index of functional impairment, kidney transplant recipients reported a quality of life that surpassed the other patient groups, although the statistical significance of these differences has not yet been examined. On many indicators, heart transplant recipients are very comparable with home hemodialysis patients and on all indicators, with the exception of ability to work, they equaled or surpassed in-center hemodialysis patients. Moreover, on all indicators but life satisfaction, they surpassed continuous peritoneal dialysis patients.

Of these results, those concerning objective quality of life are the least surprising. Clearly, patients with compromised health status have problems working and functioning that are uncommon among people in good health. What is surprising, however, is how highly all patients rate their subjective quality of life. This likely owes to

Table ES-18
Summary of Quality of Life Scores For All Patient Groups

PATIENT GROUP		UNADJUSTED OBJECTIVE INDICATORS OF QUALITY OF LIFE			UNADJUSTED SUBJECTIVE INDICATORS OF QUALITY OF LIFE		
		FUNCTIONAL IMPAIRMENT[1]	ABILITY TO WORK[2]	HEALTH STATUS[3]	WELL-BEING[4]	PSYCHOLOGICAL AFFECT[5]	LIFE SATISFACTION[6]
HEART TRANSPLANT RECIPIENTS	$\bar{X}$	1.47	57.88	9.60	11.11	5.49	5.11
	S.D.	(.772)	(49.55)	(9.01)	(2.53)	(1.32)	(1.28)
KIDNEY TRANSPLANT RECIPIENTS	$\bar{X}$	1.96	74.13	5.52	11.83	5.62	5.66
	S.D.	(1.30)	(43.95)	(7.06)	(2.72)	(1.25)	(1.62)
HOME HEMODIALYSIS PATIENTS	$\bar{X}$	2.56	59.30	9.53	11.12	5.42	5.19
	S.D.	(1.19)	(49.21)	(9.13)	(2.67)	(1.20)	(1.56)
IN-CENTER HEMODIALYSIS PATIENTS	$\bar{X}$	3.11	37.20	13.89	10.77	5.15	5.11
	S.D.	(1.57)	(48.40)	(11.24)	(2.73)	(1.31)	(1.69)
CONTINUOUS PERITONEAL DIALYSIS PATIENTS	$\bar{X}$	3.01	24.69	13.69	11.05	5.24	5.30
	S.D.	(1.34)	(43.39)	(9.71)	(2.83)	(1.26)	(1.68)
ALL DIALYSIS PATIENTS	$\bar{X}$	2.88	44.68	12.16	10.94	5.27	5.16
	S.D.	(1.43)	(49.75)	(10.54)	(2.72)	(1.26)	(1.64)
GENERAL POPULATION	$\bar{X}$	N/A	N/A	N/A	11.77	5.68	5.45
	S.D.	N/A	N/A	N/A	(2.21)	(1.12)	(1.25)

[1]Based on the Karnofsky Index, range of values = 1.0 to 10.0 where 1.0 = absence of dysfunction and 10 = maximum dysfunction (death).

[2]Ability to work, range of values = 0.0 - 100.0, where 0.0 = patients unable and 100.0 = patients able.

[3]Total Sickness Impact Profile score only, range of values = 0.0 - 100.0. Lower scores indicate better health status.

[4]Range of values = 2.1 to 14.7 where high score = positive well-being.

[5]Range of values = 1.0 to 7.0 where 7.0 = positive affect.

[6]Range of values = 1.0 to 7.0 where 7.0 = positive satisfaction.

the remarkable ability of humans to adapt to adverse circumstances, including those individuals whose lives have been seriously threatened by disease. Perhaps this has been no better stated than by the great bacteriologist, Rene Dubos, in his treatise Man Adapting:

> The components of the body machine react with the environment, in the same way as do similar components in any other living or inanimate system. But living man responds to his environment. I have intentionally differentiated between reaction and response to emphasize that human adaptation can seldom be regarded as merely the result of man's body reacting with environmental forces. In fact, man's responses are not even necessarily aimed at coping with the environment. They often correspond to an expressive behavior and involve the use of the environment for self-actualization. Health in the case of human beings is more than a state in which the organism has become physically suited to the surrounding physiochemical conditions through passive mechanisms; it demands that the personality be able to express itself creatively.
>
> Human life is thus the outcome of the interplay between three separate classes of determinants, namely: the lasting and universal characteristics of man's nature, which are inscribed in his flesh and bone; the ephemeral conditions which man encounters at a given moment; and last but not least, man's ability to choose between alternatives and to decide upon a course of action. Ideally, the goal of medicine has always been to help man function successfully in his environment--whether he is hunting the mammoth, toiling for his daily bread, or attempting to reach the moon.

In many respects, Dubos is asking people not only to think about, but to consider that which many regard as unthinkable. People are not passive objects of their environment, but are active creators of their life circumstances. When their health has deteriorated and their life has been threatened, they do what comes most naturally--adapt.

6. THE COST OF HEART TRANSPLANTATION

Given the large number of medical providers (i.e., physicians and hospitals) often associated with the care of the patient with end-stage cardiac disease (ESCD) and the wide variation in hospital accounting procedures, it is undeniably difficult to calculate the cost of a heart transplant. Moreover, the fact that a heart transplant procedure per se is only one cost associated with the treatment of ESCD is frequently overlooked. For example, even in the absence of a heart transplant, patients with ESCD incur costs associated with their medical treatment. Collectively, these costs might properly be referred to as the cost of dying. In effect, the cost of a heart transplant must be viewed as the marginal added costs that an ESCD patient incurs over and above what would have been required for his care otherwise. In this regard, it is valid to distinguish conceptually between the cost of a disease, in this case ESCD, and the cost of a transplant, which represents but a single approach to the treatment of a terminal condition.

According to the cost of illness guidelines (see Hodgson and Meiners, 1982), there are numerous categories of costs associated with illness. Chief amongst these are "direct medical costs," but also important are the following costs: (1) direct nonmedical costs--other costs borne by patients to obtain care, such as transportation costs and temporary lodging expenses, (2) indirect costs--the value of output lost because of illness, and (3) psychosocial costs--effects on the quality of life of patients. These latter three costs have been addressed above.

The six centers that participated in the National Heart Transplantation Study provided medical cost information for all their patients transplanted during the period January 1, 1978 to June 30, 1983. The information was provided for both living and deceased patients as well as patients who had been declared candidates for heart transplantation and those patients who were undergoing evaluation for a heart transplant.

Conceptually, the transplantation experience can be divided into three distinct phases. They are: (1) the pretransplant phase, including evaluation and candidacy, (2) the transplant phase, from the transplant operation to initial discharge or death, and (3) the post-discharge or posttransplant phase, including all the years since the patient's initial discharge. Costs aggregated according to these phases greatly facilitates the analysis of the data. But, despite this, several analysis complications are immediately apparent.

The first complication in cost analysis arises when it is recognized that "costs" actually differ from "charges." Conceptually, in a study such as the National Heart Transplantation Study, an estimate of the true opportunity costs of the resources used is desirable. Ideally, in well-functioning competitive markets this could be measured by the market prices of the resources used. In the U.S. hospital industry, however, the charges, which are effectively list prices for various hospital services, are unlikely to be a reliable guide to the opportunity costs of resources. Previous analyses of heart transplant costs have been deficient in this regard; they have relied on reported hospital charges. Also, unlike these

previous analyses, the intent in the National Heart Transplantation Study was to project costs to Medicare. In doing so, it was necessary to go one step further by adjusting hospital charges to "costs," as interpreted in Medicare's hospital cost-reimbursement system. How these Medicare "costs" are related to true opportunity costs is unknown, but hospital costs to Medicare are estimated by multiplying reported charges by the relevant cost-to-charge ratios and per diem costs contained in the Annual Medicare Cost Report submitted to HCFA by each hospital. In addition, to complete the cost analyses, estimates from various years are converted to constant 1983 dollars, through multiplication by a price index.

A second complication associated with the cost estimation process is derivation of non-transplant center costs incurred by patients for their medical care. Much of the cost data reported here have been derived from the medical and hospital records maintained by the transplant centers. Costs incurred by patients outside of the transplant center have been estimated using a variety of approaches.

Table ES-19 provides a summary of the parameter estimates of the medical costs of heart transplantation likely to be incurred by HCFA. Some explanation is required concerning the "base case" estimates as well as the range estimates listed as "high" and "low." The base case reflects what is assumed to be the most likely or most realistic estimate. However, numerous assumptions have been made in deriving these costs, and they are not all held with the same degree of confidence. In order to test the sensitivity of the results to alternative assumptions, sets of "lower bound" and "upper bound"

Table ES-19
Summary of Parameter Estimates for
Medical Costs to HCFA

Phase and Element	Base Case	Range Low	Range High
I. PRE-TRANSPLANT			
A. Evaluation Cost	$ 3,700	$2,700	$5,200
B. Candidacy Cost (per month)	6,151	$1,000	--
II. TRANSPLANT PERIOD COSTS			
A. Survivors	$75,085	$60,843	88,675
B. Decedents	$80,286	67,230	98,502
III. POST-DISCHARGE ANNUAL COSTS			
A. First Year:			
- Survivors	$ 9,535	--	$14,016
- Decedents	$11,810	--	$17,684
B. Second Year and Beyond:			
- Survivors	$ 6,261	--	$ 7,975
- Decedents	$ 8,911	--	$12,750

estimates are specified. The lower bound cost assumptions are those which lower the net cost of transplantation. For example, lower transplant costs and higher alternative treatment costs lower net costs. The upper bound cost assumptions are those which are likely to increase total costs. One major base case assumption is that coverage of heart transplantation will only be extended to those patients who would be Medicare-eligible under current provisions.

The pretransplant phase identified in Table ES-19 includes both evaluation and candidacy costs. The marginal cost of the evaluation at the transplant center is estimated to be $3,700. The average costs incurred during the evaluation period are much greater than this, but it is assumed that most of these costs would have been incurred by patients even in the absence of a heart transplant. The net candidacy cost per month for those patients eventually transplanted was assumed to be zero, again, on the grounds that the reported costs would have been incurred in the absence of a heart transplant. The candidacy cost estimates shown in Table ES-19, based on the experience of recipients prior to transplant, is a very important parameter. It represents the best estimates of what the monthly expenses for patient care would have been without a transplant. A potential fallacy of this extrapolation is the presumption that the recipients would have incurred as much cost had they died at their local hospitals rather than receive a heart transplant.

The costs during the transplant hospitalization were estimated at $75,085 for survivors and $80,286 for those who died

before discharge. These are weighted averages based on 1983 heart transplant volume at the centers. Although the estimates show higher average costs for those who die, this differential should not be regarded as stable. In fact, at some centers, the cost of caring for the nonsurvivors was lower than that for survivors.

The cost estimates for all years post-discharge are largely based on transplant center hospitalization records. Because of the difficulty of obtaining costs at other locations where patients may have received medical treatment, the estimates may well understate the actual costs. As shown in Table ES-19, the costs associated with dying are substantial, ranging from $9,000 to $12,000 in the year of death.

Table ES-20 presents the results of the cost-effectiveness analysis. The base case estimate is $23,478 per life year gained. Recipients were, on average, projected to have an undiscounted net life expectancy of 6.5 years; discounted (at 6 percent) net life expectancy was 4.1 years. Similarly discounted costs for the base case were $96,237 in present value terms. The cost effectiveness ratios were subjected to a sensitivity analysis with varying plausible assumptions about discount rates and the costs of alternative treatment (i.e., not receiving a transplant). The resulting ratios fell in the range of $14,000 to $30,000 per life year gained.

It should be noted that the survival probabilities (pooled across all transplant centers for all years) used in these analyses are not as favorable as the results for kidney dialysis and transplantation, where five-year <u>patient</u> survival is 52 percent for dialysis and

Table ES-20
Cost-Effectiveness Results
(Expressed in Cost per Life Year Gained)

I. BASE CASE RESULTS (20-year Time Horizon; Discount Rate = 2.5)

Period	Expected Cost	Survival Probability
0	$76,125	.800
1	8,152	.560
2	3,674	.504
3	3,306	.454
4	2,976	.408
5	2,678	.367
6	2,362	.349
7	2,244	.332
8	2,132	.315
9	2,026	.299
10	1,924	.284

Alternative Cost = $18,453

Cost per Life Year Gained = $23,478 ($96,237/4.099 years)

II. SENSITIVITY RESULTS (Cost per Life Year Gained in 1983 dollars)

	Discount Rate		
Alternative Survival	2.5	6.0	10.0
1.7 months	$21,467	$25,445	$30,258
3.0 months	$19,811	23,478	28,002
9.0 months	$14,152	16,486	19,574

59 percent for transplantation. However, five-year graft (as opposed to patient) survival for cadaveric kidney transplants is only 31 percent, and the treatment of graft rejection as well as maintenance dialysis costs can be substantial. In fact, when compared specifically with kidney transplantation with regard to cost-effectiveness, heart transplantation does not seem to be clearly more expensive per recipient from HCFAs point of view. But compared with the cost-effectiveness ratios of other medical technologies that have been evaluated, heart transplantation appears to fall at the high end of costs.

Table ES-21 shows the projected costs to Medicare over the five-year period 1985-9. Three assumptions are crucial. They are as follows: (1) the projected total volume of heart transplantation is largely a function of organ supply; (2) the proportion of recipients who are Medicare beneficiaries depends on the Medicare coverage provisions, and (3) the expected alternative cost is based on limited evidence from candidates awaiting transplant.

The base case, which assumes that current coverage provisions continue, projects the number of total recipients in 1985-9 to be 400, 440, 484, 532, and 586; Medicare-eligible recipients are then 29, 32, 35, 39, and 42, respectively. An alternative scenario--the expansion of coverage to all recipients--is shown in the second column. The third column assumes both expanded coverage (similar to kidney transplants) and a much larger number of transplants, which assumes that organ supply expands vastly.

Table ES-21
Five-Year Projections of the Costs of Heart Transplantation Coverage to HCFA (in 1983 dollars)

I. PROJECTED NUMBER OF MEDICARE RECIPIENTS

Year	Base Case (Without Expanded Coverage)	Expanded Coverage	Expanded Coverage and Higher Volume
1985	29	400	1000
1986	32	440	1100
1987	35	484	1210
1988	39	532	1331
1989	42	586	1464

II. PROJECTED COSTS TO HCFA (in millions)

Year	Base Case (Without Expanded Coverage)	Expanded Coverage	Expanded Coverage and Higher Volume
1985	2.0	$27.4	$68.4
1986	2.4	38.5	81.2
1987	2.7	37.1	92.8
1988	3.1	42.0	105.2
1989	3.4	47.4	118.5

III. PROJECTED SURVIVORS (end of year)

Year	Base Case (Without Expanded Coverage)	Expanded Coverage	Expanded Coverage and Higher Volume
1985	20	272	680
1986	38	519	1,297
1987	56	769	1,922
1988	75	1,024	2,562
1989	93	1,289	3,222

It is clear that under the base case assumptions the cost to Medicare of covering heart transplantation would be relatively small--from $2 to 3.5 million (in 1983 dollars) per year in the period 1985-9. Even under the expanded coverage, the annual cost remains less than $50 million. And the addition of expanded volume would yield costs in 1989 of about $120 million for some 3200 beneficiaries (alive at the end of the year). This is much less than the approximately $2 billion spent on the ESRD Program. Of course, the ESRD Program serves over 70,000 people, while this heart transplant group would be only about 1,300 in 1989 under the second scenario. Alternatively, per recipient expense in the ESRD Program is about $30,000 per beneficiary. For a heart transplant program, the estimated cost would average about $37,000 per beneficiary in 1989.

Finally, sensitivity analyses revealed that the projections of aggregate costs to HCFA are not very sensitive to alternative plausible assumptions about costs or survival probabilities. The major issue is how many hearts will be transplanted. As described above, current projections are that the supply available in the U.S. is very small as compared with need.

7. LEGAL ISSUES

Unlike other areas and components of the National Heart Transplantation Study, the analysis of the legal issues did not (and could not) result in quantifiable, numeric results. Rather, the intent was to focus on analyzing the relevant legal materials in order to identify important issues that would have to be addressed in an effort

to structure coverage and reimbursement policies for heart transplantation. In reading and digesting the voluminous amount of legal scholarship and case law relevant to these issues, the objective was to accomplish two major goals. First, it was necessary to identify those particular principles of law that were considered well-settled under current legal doctrines. Not surprisingly, a large number of such themes were identified. However, the application of these principles to specific fact situations, especially one as novel as heart transplantation, was not as rich a source as could be hoped for. In this situation, the second major goal was to identify what, after careful analysis and consideration, were the directions that legal thinking was taking both in the courts and the literature. As is the case with other forms of legal prognostication, the analysis presented here may be subject to criticism for misinterpretation or failure to identify critical trends in the law. However, the material presented here contains all of the factual information upon which various judgments have been based for purposes of this report. Others may choose to reanalyze the issues and suggest different conclusions. Only time and the evolution of the law will demonstrate the accuracy of findings contained in this report.

The specific objectives of the legal analysis were as follows:

- to determine the implications of special restrictions with regard to <u>whom</u> can perform cardiac transplantation, and <u>where</u> such procedures can be performed

- to consider the issues concerning the protection of the donor against premature declaration of death

- to consider various issues related to the identity of the cardiac donor
- to consider the legal basis for an individual's claim of right to a cardiac transplant
- to interpret the legal viability of strict patient selection criteria with specific reference to the Civil Rights Act of 1964 and the Age Discrimination Act of 1975
- to consider the legal implications of de facto exclusion by income level because of out-of-pocket costs for drugs, co-insurance, travel expenses, etc.
- to determine the legal implications of the Medicare program for cardiac transplantation
- to interpret the legal implications of excluding specific procedures from Medicare coverage if other procedures of comparable or less favorable cost effectiveness ratios are accepted for coverage

The material in the following sections discusses the results of the legal analyses as they relate to each of these objectives.

Restrictions on who can perform heart transplantation. The validity of restrictions on the ability of physicians to perform heart transplantation depends, to a large extent, on whether such restrictions are imposed by the government or private agency. It is clear that the federal government can restrict the ability of physicians to engage in the practice of their choice, and it can accomplish this end through any of a number of different means, including licensing and certification restrictions, and conditions on the receipt of federal financial assistance. There have been numerous challenges to the authority of a state or the federal government to regulate the practice of medicine, and nearly all have been unsuccessful. If the regulation is undertaken in a nondiscriminatory

fashion and with the appropriate due process safeguards, it will almost certainly survive challenge.

This authority is apparent when the government regulates in order to ensure that all physicians meet a particular minimum level of skill and knowledge (through imposition of training and educational requirements). It is less clear, however, that the federal government could, by administrative fiat, impose a specific limit on the number of physicians who would be permitted to perform heart transplant procedures. The resolution of this issue will hinge on the success of the physicians' arguments based on a "right" to practice medicine. Within certain limits this right does exist. But it is subject to the authority of the government to regulate for purposes of protecting the health and safety of the public.

In spite of any argument about the "right" of a physician to perform heart transplants, the government can effectively restrict access to transplant services in any number of ways without becoming involved in disputes about such rights. The most commonly employed method in health care and other areas is to condition the receipt of federal financial assistance on adherence to particular governmentally imposed standards. Thus, as a condition of receipt of other Medicare funds, the government could insist that physicians (or more likely, hospitals) not perform heart transplantations--a perfectly legitimate exercise of the federal government's perogatives.

On the other hand, the ability of private groups to restrict the arena of practice of physicians is less clear. For instance, a local medical society may be subject to antitrust liability (discussed

below) if it should engage in activities aimed at restricting the ability of an individual physician to practice medicine. Thus, such a group could not enforce a rule that prohibited otherwise qualified physicians from performing heart transplant procedures. However, recent Supreme Court decisions affirm the ability of individual hospitals to enter into agreements with individual physicians, giving them the exclusive right to perform certain services within the hospital. Presumably, a hospital could contract with an individual transplant physician to perform any and all transplants in that hospital--and physicians not a party to that agreement could not challenge it. Similarly, a hospital could make an individual decision not to perform transplants at all (as was the case with Massachusetts General Hospital in 1980), and physicians would have no recourse against the hospital.

Restrictions on where heart transplants can be performed. The ability of the government or private groups to restrict where heart transplant procedures can be performed is not as clear as similar restrictions on who can perform such procedures. The analysis with respect to private organizations is relatively straightforward, being constrained mainly by the limits of antitrust law. Thus, the hospitals in a community or a state cannot jointly arrange to divide the market for medical services by agreeing to restrict their own practices (although individual hospitals can make decisions regarding their own services). Such an arrangement would be a clear violation of the antitrust laws.

In the alternative, there have been proposals to establish regional transplant centers, both as a way to capitalize on existing skills and facilities and to restrict the number of procedures that can be performed. It is possible that the government could undertake regionalization through a very carefully tailored set of requirements for federal reimbursement for the costs of transplantation, although this still raises the possibility that other facilities would be able to qualify. Similarly, the government could establish regional transplant centers through direct legislation, as has recently been suggested. In either case, there is some question whether regionalization would violate the rights of individuals by restricting or eliminating their access to needed medical procedures. These issues are also touched on below in the discussions of de facto exclusion of individuals on the basis of income levels. The issue here is how to handle the case of the individual who is far removed from a regional transplant center and will incur added costs in seeking the treatment he or she needs, or perhaps will not be able to obtain needed treatment under any circumstances. At this time, the answer to this particular question simply is not clear, and will have to be resolved before the process of regionalization comes to fruition.

Protection against premature declaration of death. The provisions of the Uniform Anatomical Gift Act provide some protection against premature declaration of death by requiring separation of declaration of death and organ procurement. The Act prohibits the physician who makes a declaration of death from participating in harvesting the organ. The assumption underlying this provision is

that the organ procurement team has a significant interest in obtaining an organ at the earliest (and most healthy) moment, and may sacrifice the interest of the dying patient to that of the potential recipient. Except for the most unusual circumstances, this procedure provides sufficient protections for the potential organ donor.

A more important issue, however, revolves around the standard used to declare the death of a potential organ donor--the nature of the standard obviously has important implications for the point in the dying process at which an individual can be declared "dead". The most recent and comprehensive analysis of this important issue was undertaken by the President's Commission for the Study of Ethical Problems in Medicine and Biomedical and Behavioral Research and published in the volume, Defining Death. The Commission concluded:

> An individual who has sustained either (1) irreversible cessation of circulatory and respiratory functions, or (2) irreversible cessation of all functions of the entire brain, including the brain stem, is dead. A determination of death must be made in accordance with accepted medical standards.

The Commission recognized that traditional means to determine death will continue to be applied in the overwhelming majority of cases. However, in those instances in which artificial means of support require direct evaluations of the functions of the brain, the proposal would recognize the use of commonly accepted medical procedures.

It should also be plainly stated that the President's Commission did not obtain unanimity in its deliberations or in response to its work. There remains significant legal and ethical controversy over its proposals.

Protecting the identity of the heart donor. There were two major issues here that were of concern in the legal analysis component. The first involved the issue of respecting the wishes of the next of kin of an organ donor for anonymity, while balancing the wishes of the recipient to express his or her gratitude to them. This problem seems to be more one of policy and practice than law. In some cases, both recipients and next of kin of donors express a strong desire to communicate. On the other hand, there are equally as many cases in which such communication is not desired or could result in unseemly behavior. As an example, the case of the cornea donation by actor Henry Fonda, and the possibility that in such cases knowledge of the donor could lead to undesired exploitation of the donors next-of-kin and by the recipient is discussed. As a general rule, individual transplant facilities have established policies regarding this issue--a process that seems preferable to the imposition of a rigid legal requirement.

The second issue of importance here regards the protection of the identity of the potential organ donor, whose identity may be known through tissue typing. Such cases bring to the fore a set of important legal and ethical issues that remain to be satisfactorily resolved. Suffice it to say that to date the laws have been interpreted to protect the identity of such potential donors, even though it may mean the continued illness or death of a potential transplant candidate. In the case of legal interpretation, such cases have been resolved by recourse to formalistic interpretations of state law, without extensive discussions of fundamental issues of privacy under the Constitution.

Legal basis for a right to heart transplantation. If there is one issue that is clear in the law it is the principle that there is no general right to health guaranteed by the Constitution. There are conditional rights created by the legislature that "entitle" certain groups of individuals to access to certain types of health care services. However, even these are limited by the legislature's theoretical ability to dissolve such entitlements. The more important legal issues here concern whether or not an individual has a right of access to certain health care services, and upon what legal foundation such a claim is based. The resolution of these issues is not clear--under certain circumstances individuals do have a right to health care services (i.e., inmates in prison) and can force the government to provide and pay for them. As a general rule, however, there is no such right available to the general public, absent the establishment of an entitlement program that conveys the right of access to program services.

The obverse side of this question is whether the government can restrict access to a service that is available and for which the individual is otherwise able to pay. Much of the legal analysis here arises out of cases dealing with access to unconventional or unproven medical procedures, and is confounded by this context. It seems clear that an individual does not have a right, for example, to treatment with laetrile if such treatment is not generally recognized as safe and efficacious. In the face of a determination of safety and efficacy, and in the absence of other regulations prohibiting a particular treatment, there is no sound basis for suggesting that an

individual could be prohibited from seeking, obtaining and paying for a medical treatment that is otherwise available. As suggested by the series of cases dealing with abortion funding, the government has an interest in regulating certain medical procedures, and cannot absolutely preclude access to them, but it may not have any duty to provide financial assistance to help an individual effectuate his or her choice of treatment.

Viability of strict patient selection criteria. The issue of patient selection raises some important legal issues, chief among them is the focus on discrimination in the process of patient selection. The major issues involve patterns of patient selection and selection processes that may discriminate against the handicapped, ethnic or racial groups, or specific groups of individuals based on age. The validity of challenges based on these issues is greatly dependent on the nature of the patient selection process that is put in place. The most important conclusion, however, is that these considerations are essential elements to consider when making program decisions.

The most certain conclusion from the analysis of legal issues in this area is that the Age Discrimination Act of 1975 is unlikely to be used in a serious challenge to the age criteria for candidacy for heart transplantation. The exceptions that are written into the statute and regulations that allow the use of age as a determining characteristic overwhelm the purpose of the statute in preventing age discrimination. The major challenge would be to provide empirical evidence supporting the contention that advanced age is a serious contraindication for heart transplantation.

Challenges based on the Civil Rights Act of 1964 present a bit more complicated picture. It is obvious that patient selection criteria cannot be patently discriminatory on the basis of race, sex or ethnic background. The more difficult question is whether, and to what extent, such criteria result in secondary discrimination against protected groups--and what evidence can be used to establish such discrimination. The current status of the law generally requires that a law be intended as a tool for discrimination, rather than simply having the effect of discriminating--and such intentions are extremely difficult to prove. As a result, even though patient selection criteria may have the effect of discriminating against protected groups, it is unlikely that a discriminatory intention could ever be proved.

An area of potential legal importance that was identified early on in the legal analysis was the implication of handicap discrimination in the selection of patients for heart transplantation. Although the issue has never specifically arisen, it presents important questions that must be addressed. The question concerns patient selection criteria that may discriminate against handicapped individuals, a practice specifically prohibited by law. The resolution of a challenge based on handicap discrimination depends upon the ability of the medical profession to adequately demonstrate that patient selection criteria have a substantial relationship to an individual's ability to survive and benefit from treatment--in other words, is a particular criteria, such as the absence of a history of alcoholism, demonstrably related to medical fitness? Or does it

reflect a bias based on pre-conceived notions of how medical resources should best be distributed? The task is to ensure that selection criteria are in fact "medical" criteria, and that they do not incorporate aspects of patient selection based on other matters, such as "social worth".

De facto exclusion by income level. Some of these issues have been discussed above in reference to proposals to regionalize the provision of heart transplant services. And the same conclusions are relevant here. A more direct concern, however, was the issue of whether a governmentally financed heart transplant program would have to consider its potential liability for establishing participation rates that excluded certain groups on the basis of inability to pay. Although the legal ramifications of the question are complicated, the general answer is readily seen in other entitlement programs established by the federal government. Assuming that procedural requirements are otherwise met, no legal doctrine prevents the government from requiring program participants to contribute some amount to defray the expense of providing the service, even though this means that some individuals will be excluded from the program. Thus, a government heart transplant program would be free to establish rules that required financial participation--there is simply no law or policy that requires the government to provide a service to every person who might need or benefit from it.

Implications of a transplantation program for Medicare. There are two important issues that arise out of a consideration of a heart transplant program financed by Medicare. The first is a general

consideration of the implications of establishing an additional entitlement, or increasing entitlements under an existing program. The most important consideration here involves the legal ramifications should the decision ever be made to discontinue or reduce the level of benefit. It is a legal and political reality that entitlement programs, whatever their nature and content, take on a life of their own. It is an extremely difficult and time-consuming legal process that must be undertaken to eliminate an entitlement--a process that is particularly sensitive to the procedural complexity of administrative law and practice. It is far beyond the scope of this analysis to recommend any particular policy; however, it is important to point out the implications of the creation of a new entitlement or the addition of a benefit to an existing entitlement program.

A second major consideration is whether Medicare could decide to cover heart transplantation under the existing criteria for Medicare eligibility. If the current generally accepted patient selection criteria were adhered to, it would have the effect of significantly curtailing the number of individuals who would ultimately qualify for Medicare coverage for the procedure, and at the same time would limit the potential financial impact on the Medicare system. As discussed above, there is nothing in the law that prevents a government entitlement program from implementing such a decision.

Implications of cost-effectiveness determinations. The question here involves the legal implications of excluding specific procedures from Medicare coverage if other procedures of comparable or less favorable cost-effectiveness ratios are accepted for coverage.

Thus, can heart transplantation be excluded from Medicare coverage even if it is more cost-effective than other procedures that are already covered or that may be later considered for coverage? As with so many of the other questions relating to matters of administrative decision making, the answer to this question involves political rather than legal implications. It is clear that given adherence to the general requirements of administrative law and procedure, a government program has wide latitude to formulate and implement its statutory objectives. Within the context of making coverage determinations, the issue of cost-effectiveness of a procedure being considered is only one piece of information that a program may want to consider. It is certainly neither necessary nor sufficient to drive the ultimate coverage decision. In fact, in many cases the cost-effectiveness of a procedure may be legitimately irrelevant to a government program, which may be pursuing statutory objectives that conflict with cost-effectiveness.

8. ETHICAL ISSUES

The ethical analyses addressed many of the same fundamental legal issues from the perspective of ethics and philosophy. These discussions are categorized under three major headings, ethical issues concerning patient selection, the right to health care, and donors, recipients and providers.

Patient selection. In regard to ethical discussions raised by the selection of patients for transplantation, it is important to keep in mind that the process takes place within the larger discussion of the distribution of health care generally. The selection of some

patients and the rejection of others is a choice forced not only by finite conditions of persons and institutions, but also by certain cultural, social, and economic features of the health care system.

A decision for or against federal funding of heart transplantation involves what has been called a "tragic choice": in either case, the benefits and costs of the decision will be distributed in ways that inevitably lead to suffering and death for some persons. A dramatic decision to provide transplant services to anyone who needs them should not be allowed to disguise the tragedy. Regardless of whether the government funds heart transplantation, some persons will suffer adverse consequences. It is obvious that some individuals will die (who might otherwise have lived) if federal funding is not provided. If funding is provided, some individuals will not be selected for participation who might otherwise have been able to obtain a transplant. Some recipients will be disappointed in their quality of life following a transplant. Resources that may be used to fund a heart transplant program will be drawn from other sources, perhaps to the detriment of individuals who receive services or benefits from those sources. Ethicists and philosophers continue to emphasize the fact that under any sort of government policy or program, tragic decisions will be made and tragic outcomes will result.

Right to health care. Nevertheless, it is possible to take a restricted view and ask whether a treatment such as heart transplantation can be fairly distributed when it is not possible, under the constraints imposed by scarcity, to provide it to all who may benefit from it. Inevitably, however, the ethical discussions come back to

the larger issues of societal values and judgments. The contributions regarding the right to health care and the just allocation of health resources in society take a variety of theoretical approaches and offer a multiplicity of arguments. And they result in a similar set of conclusions. First, it is generally agreed that "health" (however defined) constitutes a special good both for individuals and society. Second, it is also recognized that health is an extremely complex notion that refers to a wide range of human conditions. Finally, it is acknowledged that health care itself has only an indirect relationship to "health". Thus, philosophers almost inevitably distinguish among the moral claims to (1) health, (2) health care, and (3) access to health care. It is generally recognized that the claim of right to the first category--health--is of little importance because health, as such, is beyond the reach of human activity. The second claim--health care--is too complex to realize. But the third claim--access to health care--can be the object of a moral claim.

Donors. The era of transplantation raises the ethical issue of who has moral authority over the removal of bodily organs, and the dominant ethical opinion affirms the authority of persons over control and disposition of their own bodies. The question then arises whether it is ethically necessary that a person freely donate his or her organs, or whether other parties, such as next of kin or the state, may authorize the removal of organs that the donor has not specifically authorized. In general, ethicists have not argued that voluntary donation prior to death is ethically mandatory, although some would probably maintain that an explicit refusal to donate, made prior to

death, ought to be respected. It also appears that ethicists are satisfied that next of kin can permit the removal of an organ and, in their absence, the organ could be removed.

Ethicists have also addressed the suitability of a market system for transplantable organs. Although there is apparently no unanimous opinion, the best analysis suggests that such a system would be blatantly unfair to the poor, would involve the medical profession in a process in which individuals would risk their own health out of greed, desperation or ignorance, and would ultimately increase the cost of organs to those who now receive them for free.

Recipients. Several ethical issues concerning recipients have been the focus of a great deal of discussion: informed consent, termination of life, and quality of life. In regard to informed consent, there is universal agreement that competent individuals must be accorded respect by providers of medical care, and that such respect entails seeking their consent after informing them about the risks and benefits of proposed treatment, and alternatives to that treatment proposed.

It is not at all clear that the quality of life of transplant patients is an ethical issue. Primarily because quality of life is such a vague and unbounded concept. It can be used to designate the satisfaction an individual experiences in his or her own life, or the evaluation of certain states or experiences by someone other than the individual. The more important ethical consideration is that certain classes of persons whose lives were of a certain quality, or persons whose lives were not expected to attain a certain quality as a result

of a treatment, may be systematically excluded from consideration as candidates for that treatment. The ethical problem in such a situation involves prejudicial judgment--employing irrelevant criteria to distribute benefits within society. Ethicists can do little more than warn against this danger, and attempt to provide some insight into the criteria that are relevant for certain sorts of distributions.

The concept of termination of life raises serious ethical questions: Should a patient (or some other person) have the authority to terminate treatment and life? Should such an action be considered as ethically distinct from suicide? While heart transplantation itself might be considered extraordinary, is the transplanted organ an extraordinary means of sustaining life? For many ethicists who find strong ethical arguments to justify "rational suicide", the decision to not live with a particular quality of life would be morally acceptable. To those who require a distinction between suicide and refusal of extraordinary treatment, it is the burden of life sustained by the treatment that itself renders the treatment extraordinary. Finally, those who would permit no distinction between suicide and refusal of treatment would not approve of any decision to terminate life-saving treatment.

Providers. A consideration of ethical issues surrounding providers involves consideration of two major factors: competent practice and choice of patients. If there is either an ethical or legal right to engage in the practice of medicine, it must be based on competence, and all measures judged necessary to attain competence must be followed by the practitioner. Thus, it would be ethically

absurd to claim that a general surgeon, with no particular knowledge or skill in heart transplantation, has an ethical right to practice heart transplantation. However, ethics has nothing to say about how such competence is to be attained, judged, or enforced.

Assuming a competent physician, it also asserted that a physician has a right to choose whom he or she will serve. There is no general agreement in the ethical literature that a physician's right to choose is limited by social needs. Those ethicists who affirm a strong position regarding the right to health care would limit the physician's right to refuse to treat anyone. Those who are skeptical about a right to health care would provide a wider scope for the exercise of the physician's choice.

Constraints on the autonomy of physicians to practice, to select patients or practices of their own choosing, then, can be ethically justified. Obviously, these constraints must not violate fundamental human rights, and they must be similar to those constraints justly imposed on all other participants in the society.

9. SOURCES OF PAYMENT FOR HEART TRANSPLANTATION

One of the most significant concerns of potential heart transplant recipients is who will pay their medical expenses. Transplantation is expensive and is certainly beyond the economic means of most individuals. Private insurance is the first payment source to whom patients as well as business administrators of heart transplant programs turn. It is now estimated that nearly 80.0 percent of the U.S. population is covered by private insurance, yet not all private insurers pay for or cover heart transplantation as a

standard benefit. For example, the results of a June, 1983 survey conducted by the Health Insurance Association of America (HIAA) revealed that 82.1 percent of the nation's top commercial health insurance companies are willing to pay for heart transplants under their group major medical expense plans, although many companies indicated that payment "may depend on whether certain criteria are met." Finally, as shown in Table ES-22, of the living heart transplant recipients participating in the National Heart Transplantion Study, 61.2 percent said private insurance was the primary source of payment for pretransplant expenses, 54.6 percent for transplant expenses, and 53.3 percent for posttransplant expenses.

With the exception of the transplant center, no other source played a major role in the payment of heart transplant patient expenses. Stanford University Medical Center for many years has had a large National Institutes of Health grant that assists patients in the payment of the actual transplant operation and, to some extent, posttransplant expenses. Not surprisingly, neither Medicare nor Medicaid was a major source of payment during the transplant period. However, Medicare became a more important source during the posttransplant period.

Based on these results, it is evident that private insurers have made a substantial commitment to heart transplantation, although not all companies have a formal policy that entails payment for heart transplants. Without question, a general trend has been observed in the direction of private insurance coverage for heart transplants as a nonexperimental therapy. Several major health insurers have announced

Table ES-22
Primary Source of Payment for Heart Transplantation
By Period

	TRANSPLANT PERIOD*		
SOURCE OF PAYMENT	PRETRANSPLANT	TRANSPLANT	POSTTRANSPLANT
Out-of-pocket	6.6	0.7	2.0
Private insurance	61.2	54.6	53.3
Medicare	6.6	1.3	12.5
Medicaid	3.9	1.3	3.3
Transplant center	6.6	29.6	14.5
Direct donations	3.9	2.6	2.6
Champus or VA	5.9	3.3	6.6
Other	0.7	0.7	0.7
Don't Know/Refused	4.6	6.0	4.6

*Percentages may not equal 100 percent due to rounding error.

plans to cover heart transplantation. The fact that the results of the HIAA survey are slightly inconsistent with those of the National Heart Transplantation Study survey confirms this trend. It is likely that this trend will continue unless the results of heart transplantation decline or the cost projections of private insurers for heart transplantation are exceeded. In effect, private insurers could change their policies disallowing coverage for heart transplantation.

At this time it is possible to speculate on the potential impact of private insurance on the number of persons public insurers may be responsible for covering if a favorable coverage determination is issued by the Health Care Financing Administration. In the absence of the promulgation of a full entitlement program, public insurers, (that is, the Medicare and Medicaid programs) are likely to be responsible for covering a small number of cases. A full entitlement program, of course, would bring with it a much more substantial commitment as did end-stage renal disease when Medicare benefits were extended to patients with chronic renal failure.

10. THE DIFFUSION OF HEART TRANSPLANTATION IN THE U.S.

Coverage of heart transplantation by public and private insurers is likely to be greeted by a diffusion of the procedure to other medical institutions and a concomitant increase in the number of procedures performed. Several leading heart transplant surgeons have expressed concern about the proliferation of programs and fear a possible repeat of 1968 when many inexperienced teams throughout the world undertook one or more heart transplants. To this day, few teams

likely to embark on heart transplantation have the requisite laboratory experience considered vital to the conduct of clinical heart transplantation.

Within the heart transplant community, a series of related questions are now discussed more than casually. Among them are the following: How many programs are sufficient to meet the need for heart transplantation? With the proliferation of centers in a small geographic area, how will the available donor hearts be distributed? What is the optimal number of transplants a team should perform over the course of a year? Will patient selection criteria be relaxed with an increased number of programs? What kinds of qualifications should a surgeon have to undertake heart transplantation? What medical specialties should be represented on the cardiac transplant team? Clearly, heart transplantation has the potential to alter the practice of cardiovascular surgery in the U.S. much like coronary bypass surgery has.

In an attempt to quantify the likely diffusion of heart transplantation, Battelle entered into a subcontract with the American Hospital Association (AHA) to conduct a survey of all open-heart surgery facilities in the U.S. At the time of the survey there were 648 medical centers in the U.S. with open-heart surgery facilities; 523 or 80.7 percent of these responded to the survey.

Institutions were asked to provide information on their previous experience with heart transplantation, their plans with regard to heart transplantation, the number of various types of surgical and medical procedures they performed, and their staff

distribution of various medical and surgical specialties. These data made it possible to develop a taxonomy of open-heart surgery facilities based on the extent to which they met various requirements of a model heart transplant program. This taxonomy was then used to determine whether each institution's plans concerning heart transplantation fit with their qualifications as a heart transplant center. The requirements for a cardiac transplant program had been previously formulated by the National Heart, Lung, and Blood Institute. A very weak version of these criteria were used to place all open-heart surgery facilities in one of eight "classes" (Class I - VIII). Within each class, each institution was assigned one of four "levels" (A-D), depending upon their volume of open-heart surgical procedures and cardiac catheterizations. A Class I, Level A institution meets the following requirements:

- performs 250 or more open-heart surgery procedures per year
- has an active kidney transplant program <u>or</u> has transplant surgeons on their staff <u>or</u> has a surgeon who has performed or assisted with a heart transplant
- performs 500 or more cardiac catheterizations per year
- has at least a greater than zero full-time equivalent of each of the following specialties: anesthesiology, cardiology, cardiovascular surgery, immunology, infectious diseases <u>or</u> virology, neurology <u>or</u> neurosurgery, and pathology.

Lesser institutions fail to have the proper representation of medical and surgical specialists and a lower volume of open-heart surgical procedures and cardiac catheterizations. If an institution did <u>not</u>

have a greater than zero full-time equivalent anesthesiologist, cardiologist, cardiovascular surgeon, neurologist or neurosurgeon it was automatically assigned to special class--Class 0.

The results of the analysis revealed that 151 institutions were likely to begin heart transplantation with Medicare coverage. (Another 19 institutions said they were certain to begin heart transplants with Medicare coverage.) As shown in Table ES-23, not all of these institutions are Class I and there is considerable regional variation across the country. Fifty institutions were placed in Class 0, although some of these may be more properly classified otherwise if they had supplied all the required data.

More generally, the results of the analysis show that over the next five years there is likely to be a considerable increase in the number of hospitals and medical centers in the U.S. that embark on heart transplantation. Not all of these institutions may necessarily be qualified to do so, however. In addition to the 20 active heart transplant programs, as many as 8 inactive programs will once again begin heart transplantation. Seventy-four institutions are currently considering heart transplantation, while 91 indicated that they will begin heart transplantation within the next five years. With Medicare coverage of heart transplantation, an even larger number--170--said they are certain or likely to start a heart transplant program. Based on the survey results, a variety of estimates of the potential number of programs has been made (see Table ES-24). As shown in Table ES-24, within the next five years the U.S. could easily have in excess of 100 heart transplant programs, and quite possibly close to 200.

Table ES-23
Region By Class Classification For All Institutions That Are Likely To Begin Heart Transplantation With Medicare Coverage

	CLASS									
REGION	I	II	III	IV	V	VI	VII	VIII	O	TOTAL
Puerto Rico	0	0	0	0	0	0	0	0	2	2
New England	5	0	1	0	0	0	0	0	2	8
Mid Atlantic	5	0	5	0	4	0	0	0	6	20
South Atlantic	7	0	2	0	0	0	4	1	4	18
East North Central	8	0	3	0	3	0	5	0	8	27
East South Central	3	0	1	0	2	1	2	0	1	10
West North Central	4	0	0	1	4	0	2	0	8	19
West South Central	1	0	1	0	1	0	1	1	9	14
Mountain	4	0	2	0	1	0	0	0	2	9
Pacific	6	0	3	0	3	0	3	1	8	24
TOTAL	43	0	18	1	18	1	17	3	50	151

Table ES-24
Number of Heart Transplant Programs Under Various Scenarios

PROGRAM STATUS	UNDER CURRENT CIRCUMSTANCES	IN NEXT FIVE YEARS	WITH MEDICARE COVERAGE
Currently Active Programs	20	20	20
Reactivated Programs	8	8	8
New Programs	74*	91	170**
TOTALS	102	119	198

*Assumes that every institution currently considering heart transplantation will initiate a program.

**Assumes that all those institutions that said they are likely or certain to begin a program will do so.

Using data contained in this report, it is possible to anticipate some rather significant problems in meeting the need for heart transplantation. First, it has been demonstrated that as many as 14,100 people could require a heart transplant in any given year. It was argued, however, that many of these people would not qualify for a heart transplant because of a variety of contraindications. On the very low end it was estimated that perhaps as many as 1,932 of these 14,100 would actually be accepted for a heart transplant. Second, in estimating donor supply, it was determined that at the very outside there were 17,936 in-hospital deaths that could have given rise to an organ donor. Yet, based on prevailing attitudes, it was estimated that only 9,506 organs may have been donated. Finally, based on the actual kidney donor pool, it was estimated that, at the most, 866 hearts would be available over the course of a year, but perhaps as few as 325.

Based on these data, it is possible to estimate the number of heart transplant programs that may be required under varying assumptions. If it is assumed that the optimal number of heart transplants performed per year by any one program is 21, as the survey results show, then to meet the need for heart transplantation in the U.S., as many as 671 heart transplant programs would be required, or as few as 18 (see Table ES-25). With a substantial increase in the actual donor pool, perhaps at the very most 90-100 heart transplant programs would be required. Therefore, based on the results of the survey of open-heart surgery facilities, it is estimated that, with Medicare coverage of heart transplantation, the number of heart

Table ES-25
Number of Heart Transplant Centers Based On Varying Estimates Of The Need For The Procedure And The Supply Of Donors

BASIS FOR ESTIMATE	NUMBER OF CENTERS REQUIRED ASSUMING AN AVERAGE OF 21 TRANSPLANTS PER YEAR
All Persons In Need (14,100)	671
Low Estimate Of Persons Accepted (1,932)	92
High Donor Estimate (17,936)	671
Donor Estimate Based On Attitudes (9,506)	453
High Actual Donor Estimate (866)	42
Low Actual Donor Estimate (325)	16

transplant programs may be excessive, thus, constraining the number of procedures performed per year by any one center to a below optimal level.

Despite the potential for an excessive number of heart transplant programs, it is noteworthy that Medicare has refrained from a policy of limiting coverage of particular technologies, such as heart transplantation, to restricted circumstances (e.g., to institutions offering specific services or having specialized equipment or to physicians with specific skills). The Office of Technology Assessment (1984) has concluded that "...although the notion of limited coverage has gained importance with the increasing development of sophisticated technologies that require particular expertise, the dictum of refraining from interfering with medical practice appears foremost."

E. DISCUSSION

The National Heart Transplantation Study points to the very critical need for routine health care technology assessments in the U.S. Technological innovations, whether they be drugs, devices, or surgical procedures, are being introduced at an ever increasing rate. It is evident that the implications of most of these technologies are not well understood nor even anticipated. The End-Stage Renal Disease Program of Medicare is often cited as an example of how technology can get out of hand.

As of late, health care technology assessment has attracted considerable attention among policymakers, legislators, and medical

practitioners. It is no small wonder, as many have argued that rising health care costs are largely attributable to both large (e.g., computed tomography, organ transplant procedures) and small (e.g., overuse of laboratory tests) medical technology. To curb both the use and abuse of medical technology, legislation has been introduced in Congress to provide a mechanism by which periodic assessments can be undertaken through either a private sector organization or a government supported activity. Without question, organ transplants of all types have spurred interest in health care technology assessment. Various issues that have arisen in connection with organ transplant procedures have forced legislators and policymakers to take a longer term perspective on new health care technologies.

The National Heart Transplantation Study stands as a prototypical study in the field of health care technology assessment. The Study represents the largest single health care technology assessment ever undertaken in terms of its comprehensive coverage of a diverse but very related set of issues. In terms of sheer dollars expended, however, the study is small in comparison with major clinical trials, such as the Coronary Artery Surgery Study. A clinical trial, of course, represents one approach to health care technology assessment.

The goals of technology assessments vary depending upon the agency or organization that underwrites their costs or plans to use the results. For example, a pharmaceutical or medical device manufacturer is concerned about the profit that a new drug or medical device may turn. In this regard, a technology assessment is completed at the very earliest stages of research and development and is used as part

of an overall marketing strategy. If the new drug or device does not appear profitable, it is likely to be abandoned before sizeable resources are expended to develop it. If, however, a drug or device is demonstrated to be potentially safe and efficacious, the manufacturer is likely to commit the necessary resources for its development. Once the drug or device has been developed and subjected to preliminary trials (i.e., clinical trials), public and private insurers become interested in its application. Eventually a decision will be made concerning coverage of the drug or device. In the past, such decisions have been relatively straightforward. If, for example, a device was widely used by the medical profession, it was unlikely that insurers would exclude it from coverage. The same, of course, applies to new medical and surgical procedures. In effect, medical practitioners have traditionally dictated what they felt should be covered under insurance programs. If a question was raised, usually a group of medical consultants was called together to resolve a coverage issue.

Today the situation has changed. Whereas in the past cost was rarely a consideration in coverage determinations, today this has become a key issue. More importantly, the question asked is often of the cost-effectiveness type. When questions about cost-effectiveness are raised, it becomes apparent that the medical profession is ill-equipped to make coverage pronouncements without input from nonmedical experts. Traditionally, physicians have been taught to ignore cost as an issue. If the patient's health was at stake, the physician was expected to do everything possible to care for the

patient, regardless of the cost implications. Most practitioners continue to operate in this fashion although they are finding it increasingly difficult to do so. They now find, for example, that every course of action they may choose to take is not necessarily "paid for" by the public or private insurer responsible for the patient's expenses. Thus, the medical profession finds itself confronting the uncomfortable situation of deciding what course of action to take based on reimbursement.

This entire situation becomes more complex when insurers ask the developers of new technologies to demonstrate that their technology or treatment approach is more cost-effective than already available approaches. It is not unlikely nor unusual for a manufacturer to develop a technology that is equally or more effective than a technology that is already available. However, the cost of the new technology may exceed the cost of already available technologies. If this is so, private insurers become more reluctant to pay or "cover" the new technology. Ultimately, therefore, manufacturers face a very difficult decision when deciding how best to allocate their research and development funds. The more cost-effective a technology, the more likely it will be covered. Marginal technologies, however, are unlikely to diffuse within the medical community, meaning that considerable research and development resources have essentially been lost. Consequently, it is in the best interest of manufacturers to undertake extensive health care technology assessments as part of their research and development decision making process and as part of their overall marketing strategy.

There is little question that today and throughout the foreseeable future, insurers will dictate the course of technological development. In the past developers have enjoyed this role. Manufacturers and developers may once again dictate the future of health care technology if it embarks upon the development of products after exhaustive technology assessments and have planned for the marketing of their products. Obviously, part of this marketing strategy must also include representatives from private and public insurer groups. Physicians in many respects will be a secondary concern. If insurers are convinced of the cost-effectiveness of a product, physician groups, because of favorable reimbursement, are likely to adopt the technology rather readily.

As alluded to above, one of the primary objectives of health care technology assessment is to resolve the experiment-therapy debate that unavoidably surrounds new technologies. Over the past year controversy has raged concerning the experimental or therapeutic status of various organ transplant procedures. Both private and public insurers have been reluctant to pay for various extrarenal organ transplant procedures, frequently arguing that they are experimental procedures and, thus, not eligible for reimbursement.

However, progress in medical science is not an either/or proposition. The concept of progress is dynamic rather than static. To characterize progress in organ transplantation or the application of any surgical procedure or medical innovation as being experimental <u>or</u> therapeutic can be greatly misleading. Only at the very initial stages of development can a procedure be said to be experimental and

only when a procedure has been fully integrated into medical practice can it be considered therapeutic. At either of these two extremes the status of a procedure is self-evident and undebatable. Perhaps the concepts of questionable and acceptable should be superimposed along the experiment-therapy continuum to emphasize the uncertainties that surround many medical and surgical procedures that are beyond the experimental phase but are not yet therapeutic.

The status of a procedure is also a critical determinant of research support. If a procedure is therapeutic, less funding is made available through sponsoring organizations such as the National Institutes of Health (NIH), while experimental procedures are likely to receive continued research support. In this regard, grant review committees can serve an important, although not obvious function, in determining the status of a procedure. For example, if funds for the clinical care of patients are rejected upon grant review, the implication is that the procedure is no longer experimental. Therefore, support is to be sought elsewhere for the clinical care of patients. This source of support, quite surprisingly, is either public or private insurers.

It is critical at some point to resolve the experiment-therapy dilemma. This can be accomplished by the selection of a reference therapy that is currently accepted to which the questionable therapy can be compared. The reference therapy must be of equal complexity. For example, if heart transplantation is of questionable status, it should be compared with another accepted therapy such as kidney transplantation or renal dialysis. The two treatment approaches are

compared on the basis of appropriate reference criteria such as survival and quality of life. If the questionable therapy compares well with the accepted therapy, then it is reasonable to conclude that the former is therapeutic. The results of such comparisons can then be used to formulate health policy with regard to new technologies.

One experimental technology that is directly relevant to an assessment of heart transplantation is the artificial heart. A viable artificial heart would have tremendous implications for the treatment of end-stage cardiac disease (ESCD), since the donor organ constraints could essentially be removed. Many more people would benefit from cardiac replacement and the cost of caring for ESCD patients would increase substantially. In fact, a viable artificial heart would make the treatment of ESCD directly anologous to the treatment of end-stage renal disease (ESRD).

It is obviously much too early to comment on all the implications of the artificial heart. Only four total artificial hearts have been implanted in humans with the intent to extend their survival. Once sufficient experience has been gained, however, an assessment of the artificial heart, much like that undertaken here for heart transplantation, must be performed. The need for cardiac replacement must be reassessed taking into consideration patient selection criteria that are likely to be less strict than those for heart transplant recipients. The availability of artifical heart implantation must be determined. The survival of artificial heart recipients must be evaluated, as well as their quality of life. The cost associated with the use of the artificial heart must be carefully

documented. Finally, the legal and ethical issues associated with an artificial device must be carefully considered.

Should the treatment of end-stage cardiac disease parallel that of end-stage renal disease, an obvious question concerns the extent to which lessons have been learned from the treatment of the latter. Upon a cursory review it would seem that several lessons have been learned in three areas in particular. They include: (1) patient selection, (2) patient outcomes, and (3) treatment costs. Of these, patient selection is the primary consideration because both outcomes and costs are profoundly affected by who is transplanted.

The legislative history of the ESRD program has revealed that it is very difficult to deny available lifesaving treatment to patients in need--despite relative medical and social contraindications. Thus, while a well-intended set of selection criteria can be derived, it is doubtful whether such criteria would remain unchanged with time. Poor candidates for a heart transplant today, may be among the prime candidates tomorrow. Also, patient selection criteria may be suspended due to legal and ethical considerations. Consequently, any attempt to estimate need must take into account the great potential for relaxation of patient selection criteria. Such a relaxation of patient selection criteria is likely to markedly affect the overall composition of the patient population, as was the case when Medicare benefits were extended to ESRD patients (see Table ES-26).

A second lesson learned from the ESRD experience concerns patient outcomes, not only survival, but quality of life and rehabilitation. Elsewhere Evans and coworkers (1984) have examined the impact of

Table ES-26
Social And Demographic Characteristics Of The
Hemodialysis Patient Population, 1967 and 1978

CHARACTERISTIC	POPULATION 1967, %	POPULATION 1978, %
Sex		
Male	75.0	49.2
Female	25.0	50.8
Race		
White	91.0	63.7
Black	7.0	34.9
Other	2.0	1.4
Education		
Junior high school or less	10.0	28.7
Some high school	17.0	17.2
High school graduate	27.0	28.4
Some college	20.0	18.2
College graduate	12.0	5.7
Postgraduate school	13.0	1.8
Unknown	1.0	0.0
Age, yr		
25	8.0	3.4
25-34	24.0	10.0
35-44	32.0	14.6
45-54	27.0	25.8
55	7.0	45.7
Unknown	2.0	0.5
Marital status		
Single	16.0	13.0
Married	79.0	61.8
Separated		6.3 } 25.2
Divorced	5.0	7.4 } 25.2
Widowed		11.5 } 25.2
Employment Status		
Employed	41.7	18.4
Unemployed	38.3	17.7
Disabled		53.6 } 63.8
Student	13.2	Not coded } 63.8
Retired		10.2 } 63.8
Other	0.0	0.1
Unknown	6.8	0.0

SOURCE: Evans et al., 1981.

different ESRD treatment modalities and patient characteristics on the outcomes of treatment. They confirmed that patient selection factors, as evidenced by the characteristics of patients on each of the available treatment modalities, had a substantial affect on patient outcomes. Also, as described above, as ESRD patient selection criteria have been relaxed, the outcomes experienced by patients have concomitantly declined. In other words, it does not appear that all patients treated today will have the same quality of life and longevity reported for the patient population of several years ago.

In this same regard, it is expected that more relaxed criteria for heart transplant recipients may greatly jeopardize their outcomes. As older and sicker patients are transplanted, the complication rate will increase and quality of life and rehabilitation will decline. Although a complex social, legal, and ethical dilemma, there is no means by which the quality of life of all heart transplant recipients, regardless of age and medical contraindications, can be guaranteed to be similar to that of younger patients with fewer complications.

Perhaps the most profound lesson from the ESRD experience is the importance of cost. Here cost is used in a generic sense to denote program expenditures, patient expenses, and social welfare payments. The cost of the ESRD program continues to increase at a rate that pales in comparison with earlier projections (see Table ES-27). A period of leveling-off is not yet in sight, and policymakers, legislators, and other observers are unsure what the future may hold. Whether experience with heart transplantation will be directly comparable is debatable, but in all likelihood it will be similar.

Table ES-27
Projected Medicare ESRD Patient Population, And
Annual ESRD Benefit Payments

YEAR ENDING SEPTEMBER 30	AVERAGE ANNUAL ENROLLMENT (THOUSANDS)	TOTAL BENEFIT PAYMENTS (MILLIONS)	INCOME GENERATED BY SECOND PAYOR REGULATIONS (MILLIONS)
1974	16.6	$184	
1975	25.3	327	
1976	33.2	481	
1977	40.3	654	
1978	46.7	835	
1979	53.9	1,037	
1980	62.0	1,268	
1981	68.2	1,532	
1982	72.8	1,814	
1983	76.8	2,056	
1984	80.5	2,153	$10
1985	83.7	2,337	25
1986	86.7	2,561	35
1987	89.4	2,782	45
1988	91.2	3,004	55
1989	92.2	3,220	55
1990	93.0	3,437	60
1991	93.6	3,671	65

SOURCE: Susan Kunkel, Health Care Financing Administration, May 5, 1984.

Again, program costs will mirror patient input to the system. If patient selection criteria become relaxed, and the patient case-mix more medically complex, overall expenditures are likely to rise.

Patient expenses, although not substantial at this time, may increase as public and private insurers limit reimbursement for various treatment-related expenses, thus increasing the share that patients must bear out-of-pocket. If patients are unable to return to gainful employment, as has been the case for many dialysis patients, such a shift in "expense-sharing" may be particularly devastating.

Finally, experience with ESRD vividly shows that treatment-related costs are only one part of total expenditures. Many ESRD patients become dependent on various government income maintenance programs, but even with these income supports the patients are in difficult financial straits. Again, it is uncertain whether the same would hold true for heart transplant patients. On the positive side, of all ESRD patients, successfully transplanted patients are among the best rehabilitated. Probably as a result of patient selection, 46.0 percent of kidney transplant patients as opposed to 77.0 percent of in-center hemodialysis patients receive income support through federal programs. Also, 23.2 percent of kidney transplant recipients studied by Garrison et al. (1984) had family incomes below the poverty level, as compared with 42.0 percent of in-center hemodialysis patients.

In summary, experience has been gained and lessons have been learned from the ESRD program. Although this program is often criticized, it serves as a prototype of what a catastrophic health

care program may entail, both administratively and operationally. It does have certain limitations for creating the kinds of analogies proposed here, and the extent to which these are recognized will greatly facilitate more general health planning efforts. These planning efforts, however, will most likely include increasingly frequent decisions about resource allocation and rationing. As a final note, therefore, it is important to comment upon this subject as it relates to the National Heart Transplantation Study.

Discussions regarding the high cost of medical care, in increasing numbers, now include commentaries on the possibility that the resources available for medical care must be allocated across medical care programs and ultimately be rationed among beneficiaries (Evans, 1983b; 1983c). This has led some to argue that resources allocated to heart transplantation would be better allocated to routine cardiac care or, even further, to preventive health care activities (Evans, 1984). The issue involved is extra-ordinarily complex and probably irresolvable. In this regard, regardless of how the heart transplant issue is decided, it will never be resolved satisfactorily from the standpoint of all concerned. It is clear that heart transplantation will require a sizable, although not substantial, commitment of resources. Many will argue that such a commitment is too large while those who directly benefit from such an allocation of resources will contend that it is too small.

While heart transplantation obviously raises questions of resource allocation and rationing, it is now evident, at the completion of this analysis, that the rationing or patient selection problem is the more

formidable undertaking. Given the very limited supply of donor organs, relatively few procedures would be performed, and the economic implications are minimal when the total Medicare budget is considered. Yet, the number of people who stand to benefit from heart transplantation greatly exceeds the supply of donor organs. This implies that very difficult decisions must be made as to which individuals will be permitted to benefit from heart transplantation. Ultimately, therefore, it is easier to evade than confront the resource allocation decision since payment of heart transplant procedures are, relatively speaking, a low-cost option. From a rationing perspective, however, heart transplantation is high stakes gambling in the extremist sense. Clearly, not all patients in need will benefit and financing is not the constraint. The major constraint is a resource that, at this time, cannot be mass-produced.

Should a viable artificial heart become available, however, the rationing problem would be resolved but the resource allocation problem would become more acute. The artificial heart would clearly create a dilemma of major proportions, similar to those often subjected to ethical and moral debate, yet rarely resolved. In short, an artificial heart could be mass-produced and all in need could potentially be accommodated, but to treat everyone would have nontrivial financial implications!

It is in this regard that public opinion concerning health care costs serves as a reasonable conclusion of this summary of the findings of the National Heart Transplantation Study. In a recent review of public opinion poll data, Blendon and Altman (1984)

concluded that Americans are concerned about rising health care costs but "...contrary to the views of many national leaders and economists, most Americans are not troubled by the growing share of the nation's economy that is devoted to health care. In fact, most believe that our society currently spends too little rather than too much for these services." Only 14 percent of the public think that our society is spending too much for health care, and 53 percent think we are not spending enough. Moreover, while medical technology is often criticized as being too costly, Blendon and Altman's (1984) review revealed that "Although there is support (74 percent) for regionalizing 'big ticket' technological equipment, such as nuclear-magnetic-resonance and CAT scanners, only 21 percent of Americans are in favor of rationing costly new technological approaches." More specifically, with regard to organ transplant procedures, Blendon and Altman found that fully "...90 percent of Americans favor continued development of highly expensive heart, kidney, and other organ transplantation." In short, despite its cost, it would appear that Americans feel that health care is a good investment, and transplantation appears to be one such investment.

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